

BIOTECH ROUNDTABLE |  
SCIENCE OF START-UPS

NEXT-GEN SALES MODEL |  
INVEST AND TEST

FRUGAL INNOVATION |  
AFRICA'S TRUMP CARD?

# Pharmaceutical Executive

MARCH 2015

WHERE BUSINESS MEETS POLICY

VOLUME 35, NUMBER 3

WWW.PHARMEXEC.COM

## CHROMOSOME DE-CODERS

23andMe's Andrew Page  
and Emily Drabant Conley



Pharmaceutical Executive  
MARCH 2015  
23andMe  
African entrepreneurs  
Sales survey  
Biotech RT  
VOLUME 35, NUMBER 3



## HOW COMPLETE IS YOUR PoC?

Only Covance combines a dedicated early scientific team with market expertise to deliver a more holistic Proof of Concept solution. Complete PoC™ brings together clinical and economic PoC to help you maximize molecule value and ROI.

Whether you choose to customize parts of our development services or take full advantage of the entire suite, Covance helps you get more out of your molecule.

### CALL TO LEARN MORE

The Americas +1.888.COVANCE | Europe/Africa +800.2682.2682  
Asia Pacific +800.6568.3000 | [Or go to covance.com/completepoc](http://Or%20go%20to%20covance.com/completepoc)

Covance Inc., headquartered in Princeton, NJ, is the drug development business of Laboratory Corporation of America® Holdings (LabCorp®). Covance is the marketing name for Covance Inc. and its subsidiaries around the world. © Copyright 2015, Covance Inc.

**COVANCE**  
SOLUTIONS MADE REAL®

# Me States: Is There Gold in the DNA Selfie?



**William Looney**

Editor-in-Chief

wlooney@advanstar.com

Follow Bill on Twitter:

 @BillPharmExec

**TODAY'S PATIENT MOVEMENT IS ON THE MOVE**, with an expanding, play-it-forward agenda grounded in the many new obligations that system reform now places on the individual consumer of healthcare: as an active, co-equal partner to the clinician in successful diagnosis and treatment, and—most importantly—as a direct contributor to financing the cost of that care.

**B**ut less is known about the growing prominence of patient organizations in the crowded and intensely competitive business of scientific research. Among other things, the movement has embraced a new model—venture philanthropy—that strives to go places where risk-averse private enterprise fears to tread. The biggest and latest example is the Cystic Fibrosis Foundation's sale of its royalty rights to portions of Vertex Pharmaceutical's CF portfolio for the whopping sum of \$3.3 billion, which the Foundation will use to finance what it calls "daring opportunities for a life-long, permanent cure to this disease."

That news prompted *Pharm Exec* to cast around for similarly transformative models that fuse patient awareness and activism with a business objective. That led us to our lead feature this month: an in-depth look at the patient genome partnering advocate 23andMe, which has re-emerged from a period of bad karma with the FDA to ink some potentially groundbreaking and mutually profitable collaborations with big Pharma. That inauspicious start with regulators, which resulted in an FDA ban on 23andMe's first product, a genetic test kit for patients, proved a useful corrective to the company's initial broad lens business model, with a script written by A-list veterans of the open-access Googleplex but no fixed choreography to channel what patients might practically do as consumers of healthcare services, once they obtained information to their own genetic future. There was a risk element around inappropriate medical treatments that no amount of gloss about patient empowerment could conceal, a fact that both the FDA and 23andMe have quietly taken to heart.

Two years on, 23andMe stands out as a fast-maturing enterprise with an adaptive learning culture. Indeed, Senior Editor Casey McDonald's profile highlights how quickly the company's leadership has regrouped around a commitment to data mining as a source of researchable insights to a healthcare business whose survival now depends on treating patients not as "covered lives," but as active, choosy consumers—and as viable research subjects for industry, too.

Specifically, 23andMe's remit now sees real synergies between drugmakers, academic researchers, and the activist patient. Bringing 23andMe's motivated consumers interested in research into contact with the business side of genomics can expand the supply of useful data for industry and help drive progress in biomedical research. It's a simple proposition, with 23andMe facilitating the entry of self-selected, re-contactable individuals into industry studies for which they have relevant genetic markers, by offering such patients genomic and genealogical services to keep them interested and involved over time. The risk of consumers making poor medical choices from disaggregated genetic information has been addressed, with a scrubbed and refined counter message: information and the services that surround it need to be precisely targeted and supported for analysis and interpretation; only then can these be productively applied to the goal of improving personal health and well-being.

With signed collaboration agreements in hand, Pfizer, Roche, and other industry giants with the money to spend on the hypothetical now seem to be buying in to the adapted 23andMe model, at least for a test drive. Self-interests are converging. The industry's voracious needs for data to help generate targets and validate the growing number of test compounds derived from de-coding of the human genome shows no signs of slack. There is a ready market waiting for anyone who can fill the gap with willing, accessible subjects.

Of course, only time will tell if this 21st century proposition for a struggling 20th century research system finds its roots. To us at *Pharm Exec*, there are two relevant questions.

The first relates to standards: are all genomic sequencing technologies created equal—and are all genetic interpretations equally valid?

The second is more basic: will the personal genomics platform that 23andMe is building lead to measurable improvements in treatments and outcomes, or end up as just another fad-dish exercise, involving a few interested people willing to pay to send a sample of their own spit through the mail?

Stay tuned.

**Pharmaceutical Executive's 2015 Editorial Advisory Board is a distinguished group of thought leaders with expertise in various facets of pharmaceutical research, business, strategy, and marketing. EAB members suggest feature subjects relevant to the industry, review article manuscripts, participate in and help sponsor events, and answer questions from staff as they arise.**

**Murray L. Aitken**

Senior Vice President,  
Healthcare Insight,  
IMS Health

**Stan Bernard**

President,  
Bernard Associates

**Frederic Boucheseiche**

Chief Operating Officer,  
Focus Reports Ltd.

**Joanna Breitstein**

Director, Communications,  
Global TB Alliance

**Drew Bustos**

Vice President, Global Communications,  
Cegedim Relationship Management

**Don Creighton**

Senior Director, Market Access,  
PriceSpective, an ICON Company

**Rob Dhoble**

CEO,  
Adherent Health

**Bill Drummy**

CEO,  
Heartbeat Ideas

**Les Funtleyder**

Portfolio Manager,  
Esquared Asset Management

**John Furey**

Global Franchise Head Vaccines,  
Baxter Healthcare

**Terry Hisey**

Vice Chairman,  
Nat'l Sector Leader, Life Sciences,  
Deloitte

**Michele Holcomb**

Vice President Corporate Strategy,  
Teva Pharmaceuticals

**Bob Jansen**

Principal Partner,  
Zensights LLC

**Kenneth Kaitin**

Director & Professor,  
Center for the Study of Drug Development,  
Tufts University

**Clifford Kalb**

President,  
C. Kalb & Associates

**Bernard Lachappelle**

President,  
JBL Associates

**Rajesh Nair**

President,  
Indegene

**Daniel Pascheles**

Vice President,  
Global Business Intelligence,  
Merck & Co.

**Elys Roberts**

President,  
Ipsos Healthcare North America

**Alexander Scott**

Vice President, Business Development,  
Eisai Corp. of North America

**Sanjiv Sharma**

President,  
InflexionPoint LLC

**Michael Swanick**

Global Practice Leader Pharmaceuticals  
and Life Sciences, PwC

**Mason Tenaglia**

Managing Director,  
The Amundsen Group, an IMS Company

**Peter Tollman**

Senior Partner, Managing Director,  
Boston Consulting Group

**Al Topin**

President,  
Topin Associates

**Bill Trombetta**

Professor of  
Pharmaceutical Marketing,  
St. Joseph's University Business School

**Joseph Truitt**

Senior Vice President and  
Chief Commercial Officer,  
Achillion Pharmaceuticals

**David Verbraska**

Vice President, Worldwide Public Affairs  
and Policy,  
Pfizer

**Albert I. Wertheimer**

Professor & Director,  
Pharmaceutical Health Services Research,  
Temple University

**Ian Wilcox**

Vice President,  
Hay Group

**Peter Young**

President,  
Young & Partners

# Pharmaceutical Executive

VOLUME 35, NUMBER 3

VP OF SALES & GROUP PUBLISHER	TEL [732] 346.3018
Russ Pratt	rpratt@advanstar.com
EDITOR-IN-CHIEF	TEL [212] 951.6735
William Looney	wlooney@advanstar.com
MANAGING EDITOR	TEL [732] 346.3022
Michael Christel	mchristel@advanstar.com
SENIOR EDITOR	TEL [212] 951.6635
Casey McDonald	cmcdonald@advanstar.com
EUROPEAN & ONLINE EDITOR	TEL 011 44 [208] 956.2660
Julian Upton	jupton@advanstar.com
SPECIAL PROJECTS EDITOR	TEL [212] 951.6742
Marylyn Donahue	mdonahue@advanstar.com
COMMUNITY MANAGER	TEL [732] 346.3022
Miraj Barodia	mbarodia@advanstar.com
ART DIRECTOR	TEL [218] 740.6411
Steph Johnson-Bentz	steph.johnson@media.advanstar.com
WASHINGTON CORRESPONDENT	TEL [301] 656.4634
Jill Wechsler	jwechsler@advanstar.com
EDITORIAL OFFICES	TEL [212] 951.6600
641 Lexington Avenue, 8th floor	FAX [212] 951.6604
New York, NY 10022	<a href="http://www.pharmexec.com">www.pharmexec.com</a>
SALES MANAGER-EAST COAST	TEL [732] 346.3054
Mike Moore	mmoore@advanstar.com
SALES MANAGER-MIDWEST, SOUTHWEST, WEST COAST	TEL [847] 283.0129
Bill Campbell	wcampbell@advanstar.com
SENIOR PRODUCTION MANAGER	TEL [218] 740.6371
Karen Lenzen	klenzen@advanstar.com
AUDIENCE DEVELOPMENT MANAGER	TEL [218] 740.7005
Rochelle Ballou	rballou@advanstar.com
REPRINTS	877-652-5295 EXT. 121
	bkolb@wrightsmedia.com
	Outside US, UK, direct dial: 281-419-5725. Ext. 121
CLASSIFIED SALES & RECRUITMENT	TEL [440] 891.2793
Tod McCloskey	tmccloskey@advanstar.com
DIRECT MAIL LISTS	TEL [440] 891.2773
Tamara Phillips	tphillips@advanstar.com

JOE LOGGIA, *Chief Executive Officer*; TOM EHARDT, *Executive Vice-President, Chief Administrative Officer & Chief Financial Officer*; GEORGIANN DECENZO, *Executive Vice-President*; CHRIS DEMOULIN, *Executive Vice-President*; REBECCA EVANGELOU, *Executive Vice-President, Business Systems*; JULIE MOLLESTON, *Executive Vice-President, Human Resources*; MIKE ALIC, *Executive Vice-President, Strategy & Business Development*; TRACY HARRIS, *Sr Vice-President*; DAVE ESOLA, *Vice-President, General Manager Pharm/Science Group*; MICHAEL BERNSTEIN, *Vice-President, Legal*; FRANCIS HEID, *Vice-President, Media Operations*; ADELE HARTWICK, *Vice-President, Treasurer & Controller*

©2015 Advanstar Communications Inc. All rights reserved. No part of this publication may be reproduced or transmitted in any form or by any means, electronic or mechanical including by photocopy, recording, or information storage and retrieval without permission in writing from the publisher. Authorization to photocopy items for internal/educational or personal use, or the internal/educational or personal use of specific clients is granted by Advanstar Communications Inc. for libraries and other users registered with the Copyright Clearance Center, 222 Rosewood Dr. Danvers, MA 01923, 978-750-8400 fax 978-646-8700 or visit <http://www.copyright.com> online. For uses beyond those listed above, please direct your written request to Permission Dept. fax 440-756-5255 or email: [mcannon@advanstar.com](mailto:mcannon@advanstar.com).

UBM Advanstar provides certain customer contact data (such as customers' names, addresses, phone numbers, and e-mail addresses) to third parties who wish to promote relevant products, services, and other opportunities that may be of interest to you. If you do not want UBM Advanstar to make your contact information available to third parties for marketing purposes, simply call toll-free 866-529-2922 between the hours of 7:30 a.m. and 5 p.m. CST and a customer service representative will assist you in removing your name from UBM Advanstar's lists. Outside the U.S., please phone 218-740-6477.

PHARMACEUTICAL EXECUTIVE does not verify any claims or other information appearing in any of the advertisements contained in the publication, and cannot take responsibility for any losses or other damages incurred by readers in reliance of such content.

PHARMACEUTICAL EXECUTIVE welcomes unsolicited articles, manuscripts, photographs, illustrations, and other materials, but cannot be held responsible for their safekeeping or return.

To subscribe, call toll-free 888-527-7008. Outside the U.S. call 218-740-6477.



2011 Neal Award  
Winner for  
"Best Commentary"



# Omnicare

Specialty Care Group

RxCrossroads • Advanced Care Scripts



*Patient Solutions.*  
**COMMERCIAL SUCCESS.**

Innovative programs that integrate *access, affordability* and *adherence*.



**Brand Support  
Services**



**Specialty  
Pharmacy**



**Third Party  
Logistics**



**Program  
Pharmacy**

To learn more about **Omnicare SCG** contact **877.901.3711** or visit **[www.omnicarescg.com](http://www.omnicarescg.com)**.

## 6 Contents



## Chromosome De-Coders

Casey McDonald, Senior Editor

*Pharm Exec* profiles patient DNA partnering advocate 23andMe, which recently struck data deals with a pair of big Pharmas—moves that are dogged by three key questions amid the overarching quest to put patients at the center of the modern research-based enterprise.

# 16

On The Cover: Andrew Page, president of 23andMe, and Emily Drabant Conley, the company's director of business development (photo credit: Joseph Schell).



### Executive Roundtable

## Behind the Boom in Biotech

William Looney, Editor-in-Chief

*Pharm Exec* convenes a Roundtable discussion with nine company aspirants for leadership in the hard science of small biotech.

# 24

### Emerging Markets

## Africa's Geography of Ideas

William Looney, Editor-in-Chief

Start-up and R&D leaders from South Africa share their pursuits for therapeutic diversity and healthcare innovation back home.

# 34

### Commercial Strategy

## Selling Strategies of Tomorrow: Invest & Test

By Rick Edmunds, Rolf Fricker, Stephan Danner, and Nelia Padilla, PwC

A new survey by PwC reveals a strong need to fully embrace alternative commercial methods—not in a blind frenzy but through careful evaluation of which new sales strategies have true innovative potential.

# 40

## NEWS & ANALYSIS

### Washington Report

#### 11 Will FDA Fixes Spur Innovation?

Jill Wechsler, Washington Correspondent

### Global Report

#### 14 STAMPing on MAPPs: Regulatory Modernization Concerns in Europe

Reflector, Brussels Correspondent

## STRATEGY & TACTICS

### Managers Notebook

#### 44 Our Love Affair with Buzzwords

By Timothy White

## INSIGHTS

### From the Editor

#### 3 Is There Gold in the DNA Selfie?

William Looney, Editor-in-Chief

### Back Page

#### 46 Cholesterol is Back: Market Drama Awaits

Casey McDonald, Senior Editor



Can we afford the medicine?

How will I get my medicine?

Will she take the medicine?

Who will help us?

How will my medicine affect me?

**FOR YOUR PATIENT**

## PRODUCT ACCESS SUPPORT SERVICES

Minimizing Barriers, Optimizing Access.

TrialCard provides strategic guidance and comprehensive solutions to optimize stakeholder experience and enhance outcomes.

The needs of the patient and the provider are the cornerstones of TrialCard Market Access Solutions, **Product Support Services Programs.**



**For the patient,** our process streamlines access to medications by resolving the various challenges that can create barriers to patient access, adoption, and utilization.



**For the provider,** we clear the path to access medications by resolving product reimbursement hurdles, minimizing staff burden and confusion.



**For the brand,** we provide strategic guidance that employs customized, yet flexible, programs with flawless execution that leads to positive brand experiences for patients and providers.

**For more information, please contact:**

John Van Johnston | Director, Business Development Product Access Support Services  
p: 919.415.3131 | e: [jvanjohnston@trialcard.com](mailto:jvanjohnston@trialcard.com)

**trialcard**<sup>®</sup>  
[www.trialcard.com](http://www.trialcard.com)



## this month on PharmExec.com

### Top Stories Online



#### The 7 Deadly Sins of Product Launches

February issue online  
Stan Bernard  
[bit.ly/1BhpNbG](http://bit.ly/1BhpNbG)

#### 2015 Pipeline Report

November issue online  
Josh Baxt  
[bit.ly/1vam3jC](http://bit.ly/1vam3jC)



#### Pharm Exec's Industry Outlook

January issue online  
Casey McDonald, Julian Upton  
[bit.ly/14aikfE](http://bit.ly/14aikfE)

#### Launch Blueprint: Steps to Success

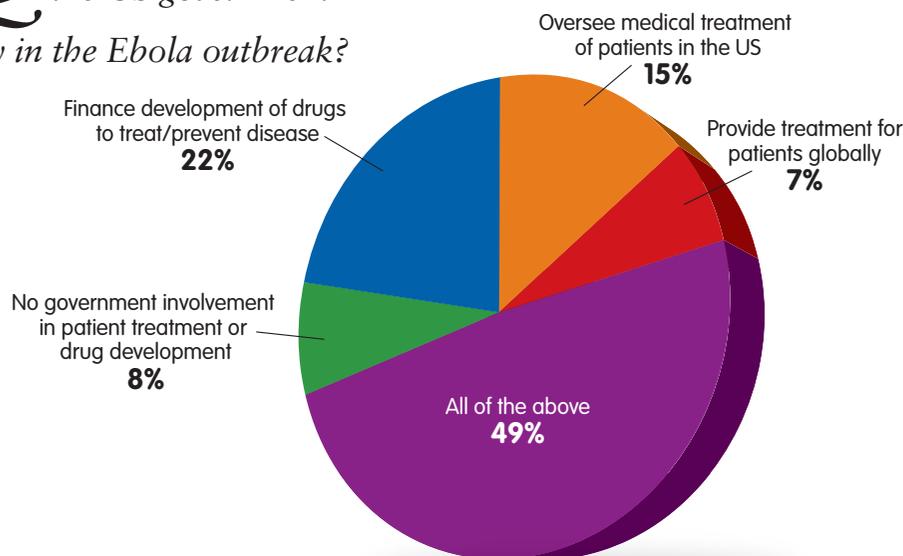
February issue online  
William Looney  
[bit.ly/186VDMm](http://bit.ly/186VDMm)

Most-read stories online:  
Jan. 25 to Feb. 24, 2015

### Data Point

**Q:** *What role should the US government play in the Ebola outbreak?*

Poll data courtesy of online *Pharm Exec* readers between Dec. 1 and Dec. 30, 2014



### Readers Weigh In

*Excellent assessment as to the purpose of healthcare reform* and how one pharma product delivers on the pursuit of value for patients and the healthcare system. Hopefully and ideally the broader media will also start to share these same insights.

Brad, 10/22/14

"The 'Value' of Rx's Under Obamacare"

[bit.ly/1DbpL1X](http://bit.ly/1DbpL1X)

*If the industry leadership does not go toe-to-toe* with the media on this vital issue, Obamacare and all its alternatives to come in the future will trend toward rationing of treatment options as they do in Canada and the EU. The insurers and the government providers will not help you. They may be your customers, but they are not your friends.

Mike Pucci, 10/9/14

"The 'Value' of Rx's Under Obamacare"

[bit.ly/1DbpL1X](http://bit.ly/1DbpL1X)

*The move to PharmD will be great* for those able to take advantage of it. Right now, I believe all of the PharmD programs are full-time six-year programs. A current pharmaceutical rep might not be able to take the time off or take on the six-figure investment.

Life After Pharma, 9/4/14

"2014: End of the Road for the American Rx Salesperson?"

[bit.ly/1zAxCSW](http://bit.ly/1zAxCSW)

### Keep in Touch!

Scan here with your smartphone to sign up for weekly newsletters



Coming soon to  
**PharmExec.com**



**Compliance Roundup**  
*Pharm Exec's* annual report highlights the latest global strategies and legislation enforcing pharmaceutical compliance and ethics.

# Announcing Market Access Solutions for Your Patient



## TrialCard Market Access Solutions Provide Positive Outcomes at All Stages of the Patient Journey

### Your brand can leverage:

- TrialCard's Hub solution, which delivered **50% year-over-year revenue growth** over the past 2 years.
- Analytical insights from **53 million prescriptions**, written by **530,000 prescribers**, filled by 21 million patients.
- Fewer abandonments and switches through adherence messaging customized to each patient's prescription schedule.
- Up to **50% NRx lift** tied to spillover from prescribers participating in a co-pay program.
- **1.2 million healthcare-focused interactions** by our award-winning customer experience center each year.

#### For more information, please contact:

John Van Johnston | Director, Business Development Product Access Support Services  
p: 919.415.3131 | e: jvanjohnston@trialcard.com

**trialcard**<sup>®</sup>  
Access. Acquisition. Adherence.

---

MARKET ACCESS SOLUTIONS

---



[www.trialcard.com](http://www.trialcard.com)

## SPOTLIGHT ON AVANT HEALTHCARE THROUGH THE EYES OF LEO FRANCIS, PhD

# OUR

# PROMISE



*Leo Francis, PhD  
Chief Strategy Officer,  
Avant Healthcare*



If you spend any time in the healthcare marketing and advertising world, chances are you have met or know of Leo Francis, PhD. His background in education, research, medical content design, and commercial marketing gives him a unique perspective on the pharma world and where it's heading.

After working and traveling abroad, holding positions at New York agencies, and being recognized in 2010 and 2012 as one of the 100 most inspiring leaders in the life sciences industry by *PharmaVOICE*, Francis accepted a position as Chief Strategy Officer for medical marketing communications company Avant Healthcare.

Although his home is in New Jersey, Francis found something intriguing about the Carmel, Indiana-based company—something intriguing enough to pull him away from his time and his successful track record at the big agencies out East.

So, what drew him to a boutique company in the heart of the Midwest? Francis says it was “an authenticity that permeates the work of the agency and its people.” It’s that culture Francis values about working for Avant Healthcare, citing the corporation’s core values—H4S (healthy, honorable, humble, hungry, and smart)—as a touchstone for understanding how the company operates.

In addition to the culture, Francis is also impressed with its longevity: “Avant Healthcare has been in business for more than 21 years, and our first client company is still with us today. Additionally, the median tenure of our staff is exceptional for this industry. It says a lot for a company’s commitment to excellence if both employees and clients want to stick around for the long term.”

So, if Avant Healthcare is doing such amazing work, why have you never heard of it? Francis credits geography and a humility amongst its staff as the reasons Avant Healthcare isn’t more well-known. “We go about our exceptional work and take care of our people without pomp or ceremony. Now that we’re continuing to build into Chicago, the West Coast, and the East Coast, more people are beginning to take notice.”

In his role as Chief Strategy Officer, Francis has to deal with an ever-changing environment driven by client requests, increasing regulations, and changing models of healthcare delivery. He recognizes the staying power of Avant Healthcare as a result of its status as “a hybrid agency that brings the best of science, creativity, marketing strategy, and customer service, illustrated by our internal capabilities and diversity in our client base.”

As Francis continues his work at Avant Healthcare, he knows the road ahead is challenging but says his people are up to that challenge. “The industry is changing, often at a rate we can’t control, yet that change is something we can harness and embrace. No matter the role, the people at Avant Healthcare are making a difference in the lives of patients around the world.”

Are you looking for a dynamic and fulfilling place to work? Do you need an agency with the skills and experience to execute your product strategy? Visit [AvantHC.com](http://AvantHC.com) to learn more about the extensive opportunities available at Avant Healthcare.

### Leo’s Top 5 Things Clients Should Consider When Selecting an Agency

1. Strategic thinking with proactive recommendations about your business.
2. Breakthrough creativity woven into deliverables.
3. Scientific expertise applied to medical education experiences.
4. How the agency approaches innovation.
5. Provide everything you need and nothing you don’t.

Follow Avant Healthcare  
on Twitter @AvantHC



# Will FDA Fixes Spur Innovation?

**Congress, Obama offer strategies to reduce regulatory roadblocks to new breakthroughs**

The productivity and efficiency of the US biomedical research enterprise is undergoing a broad reexamination, including how FDA regulation serves to bring—or block—new medicines from the market. The announcement of FDA Commissioner Margaret Hamburg's departure at the end of January further heightens the focus on recent agency accomplishments and options for change.

## 'Precision' & 'Cures'

FDA's role in encouraging new medical technology appears in President Obama's "Precision Medicine Initiative," a \$215 million program to create a massive database of patient genomic information to support future biomedical research. The proposal provides \$130 million for the National Institutes of Health (NIH) to develop the data system, \$70 million for the National Cancer Institute to identify genomic factors important for developing effective oncology treatments, and \$10 million for FDA to devise new strategies for regulating next generation sequencing (NGS) technologies.

Despite its high-profile announcement, the White House plan faces serious hurdles. Linking together data on one million patients held in diverse systems is no easy task, particularly with the need to protect patient privacy while

facilitating researcher access to the information. The administration's budget plan for 2016 highlights the "Precision" program, along with proposals to bolster research on new drugs to combat antibiotic resistance, and to modestly increase NIH and FDA funding.

---

*Linking together data on one million patients held in diverse systems is no easy task, particularly with the need to protect patient privacy while facilitating researcher access to the information*

---

The Obama plan comes on the heels of the "21st Century Cures Act," a massive document offered by House Energy & Commerce Committee chairman Fred Upton (R-Mich) designed to launch a broad discussion about multiple reform proposals. The package features numerous patent and exclusivity provisions that aim to encourage private sector investment in biomedical research activities, along with measures to streamline clinical trial operations, reform oversight of medical devices, and expand incentives for antibiotic development.

The "Cures" package also proposes to loosen controls on drug marketing. One measure would permit a "one-click" policy that simplifies how pharma

companies can provide links to risk information when discussing approved products via Twitter, Facebook and other Internet platforms. And it alters the Sunshine Act so that journal articles and medical textbooks, as well as funding of independent continuing medical education, would not have to be reported as "gifts" to prescribers. Compassionate access to not-yet-approved therapies for severely ill patients gets a nod by requiring pharma companies to be more "transparent" about access programs and forming a new task

force to recommend further access reforms.

The unveiling of Upton's 400-page "discussion draft" was muted, however, by an absence of the bipartisan support that has been important to building interest in this broad initiative, and by its failure to mention any funding strategies for its many programs. Rep. Frank Pallone (D-NJ) expressed disappointment with the plan, and "Cures" co-chair Diana DeGette (D-Colo.) withheld her endorsement. Health and biopharma organizations issued perfunctory statements supporting the effort, and DeGette left the door open for continuing negotiations to reach bipartisan consensus.

## Senate weighs in

The House Cures proposal could gain from a related ini-



Jill Wechsler is Pharmaceutical Executive's Washington correspondent. She can be reached at [jwechsler.advantstar.com](mailto:jwechsler.advantstar.com).





## POPOPIA

A condition commonly caused by working with Fingerprint. A heightened level of broad thinking and innovative solutions can lead to eye-popping responses in those new to partnering with a high-octane, truly integrated, independent marketing agency.

CALL ED MITZEN 518.488.8304 OR VISIT US AT [fingerprintmarketing.com](http://fingerprintmarketing.com)

SARATOGA SPRINGS, NY | VILLANOVA, PA | SCOTTSDALE, AZ

*fingerprint*  
Create  
Something  
Original

# STAMPing on MAPPs: Is Europe trapped again by division?

**New EU group could ultimately end up handcuffing much-needed efforts to modernize drug authorization**

Europe is at risk, yet again, of falling victim to timidity born of its divisions. Its indecisive responses to the challenges to its single currency, or to Russia's erosion of international law, are mirrored in its approach to medicines innovation, too.

Everyone in the pharmaceutical sector is now starting to get to know about MAPPs, the acronym for a convenient concept—"medicines adaptive pathways to patients"—that is now being explored in many different ways by pioneering spirits across the world. Basically, it's a matter of getting good new medicines to patients more quickly—or, to put it another way, of modernizing regulatory systems to take account of advances in biology. The discussions now taking place in Europe, the US, Singapore, and Canada reflect a common concern to see valuable innovations put to use sooner, and more judiciously, than is permitted by the current frameworks for authorizing and paying for medicines.

## Pacing the discussion

On MAPPs, Europe has, as is also so often the case, generated much of the leading thinking on how this clash between new and old might be resolved. Most prominently, the European Medicines Agency (EMA)

is running a pilot with a dozen real medicines in the early stages of development, to identify what procedures need to change—among regulators and drug sponsors—to stand a chance of making real improvements in the system. Several European countries are also experimenting

at national level with their own approaches—the UK is running an early access to medicines scheme; France, Belgium, and Spain all offer some examples of early authorization.

## STAMP swoops in

But just as the term MAPPs attains currency in the world of medicine, the European Union (EU) has come up with STAMP—a new expert group ostensibly intended to assist the process, but which, in its current manifestation, appears just as likely to suffocate it. STAMP stands for "safe and timely access to medicines for patients"—which sounds comfortingly like the same concept. On paper, its mandate, to provide advice and expertise to the European Com-

mission on the use that can be made of EU pharmaceutical rules and related programs and policies, looks constructive. Its planned activities appear broadly consistent with the objectives of MAPPs: "STAMP will exchange views and information about the experience of member states, examine national initiatives, and identify ways to use more effectively the existing EU regulatory tools, with the aim to further improve safe and timely access and availability of medicines for patients."

But take another look at that last sentence. Ignore the well-intentioned phrase about

---

*At the heart of the discussion is how to shift away from the straitjacket created by 50 years of a defensive approach to drug regulation*

---

improving access, and focus on the preceding phrase: "to use more effectively the existing EU regulatory tools." Innocuous? Plausible? Perhaps at first glance. But in the context of what is at stake in the discussions on MAPPs, potentially ruinous. If the EU's most strategic response to this effervescence of thought about a fundamental process in healthcare is to set up a group to see how the existing rules and tools can be used, it is missing the point of the exercise.

## No arguing the 'alternatives'

There is a very obvious reason why a more open-minded response is called for. Alternative pathways are alternatives. And the need for alternatives has already been clearly established by

work that is underway—and has been underway for at least half-a-decade now—on tackling the mismatch between new science and traditional regulation. This is how the EMA sees its own challenge: “The concept of adaptive pathways foresees either an initial approval in a well-defined patient subgroup with a high medical need and subsequent widening of the indication to a larger patient population, or an early regulatory approval (e.g., conditional approval) which is prospectively planned, and where uncertainty is reduced through the collection of post-approval data on the medicine’s use in patients.”

This is how the EU’s own Innovative Medicines Initiative—the multi-billion public-private partnership on drug research—sees it: “MAPPs refers to a flexible development and access pathway within the current regulatory framework that maximizes the positive impact of new medicines on public health by balancing timely access for patients with the need to provide evolving information on benefits and risks. It requires the early marketing authorization of a product focused on a well-defined and targeted population identified by predictive preclinical and clinical evidence as well as various sources of real-world evidence. It implies a clear safety and efficacy profile and may integrate a number of elements such as adaptive clinical trial design, patient-centric benefit/risk assessments, and the continuous evaluation of a therapy as new evidence (including real-world evidence) becomes available. MAPPs, therefore, relate to the entire life cycle of a medicine

from development, through licensing to patient access (pricing/reimbursement and health-care delivery).”

At the heart of the discussion is how to shift away from the straitjacket created by 50 years of a defensive approach to drug regulation—that cumulative, but inevitably futile, attempt to eliminate all risk by multiplying safety testing—and accept that the performance of a medicine may be more effectively measured by other procedures. New science opens up the possibility for changing the focus from a confrontational system of extensive pre-release trials and a one-off decision on what a medicine will do. It permits a more collaborative relationship based on continuous assessment by all involved parties, using more predictive mechanisms and better post-release monitoring, to obtain better outcomes. But it presupposes a readiness to look again at the current arrangements for balancing the need for prior evidence with the need for early patient access.

That is why sticking to the rules as a precondition for reflection is not likely to impel the sort of radical thinking and constructive development that a better deal for patients depends on.

### Radical thinking required

If the heart of the discussion is the balance of evidence and access, the muscle of the debate could be described as a new balance of access and affordability—which also has to be taken into consideration in advancing MAPPs. The fastest procedures for authorizing a medicine are no use unless patients can actually obtain the medicine—and

that is a function of the distinct national systems for payment or reimbursement for prescribed drugs. So making a reality out of MAPPs requires radical thinking, too, about how to bridge that gap. Here, too, however, STAMP appears to stamp even more firmly on EU initiative.

“It was clarified that the role of the group,” reads the record of the first STAMP meeting, held in early 2015, “is not to provide advice for the revision of the basic acts [of EU pharmaceutical legislation]. In addition, health technology assessment, pricing, and reimbursement will not be the focus of STAMP.” At a stroke—or perhaps with a sudden exertion of downward pressure—the EU rejected out of hand any talk of a review of the pricing and reimbursement arrangements that most critically govern actual patient access. The reluctance—nay, the downright refusal—to countenance any discussion of the economics is a consequence of Europe’s Achilles’ heel in the health sector.

### Dueling pursuits

Each member state retains absolute autonomy over health spending—and by implication, drug pricing and reimbursement. This autonomy is a longstanding and stoutly defended position of the member states (as is also, by no small coincidence, the case for foreign policy and for national economic management). It satisfies their own sense of responsibility. But it may, at the same time, amount to irresponsibility in terms of pursuing common goals—as the respective fates of the Euro, Ukraine, and innovative medicines are likely to demonstrate. **PE**



**SIDE BY SIDE WITH OUR CLIENTS  
WE STRIVE TO MOVE  
DRUG DEVELOPMENT FORWARD.**



#### **EFFECTIVE AND SUSTAINABLE SOLUTIONS**

At PRA Health Sciences, we understand the dynamic nature of clinical development and the needs of sponsors. Our Strategic Solutions division has created a customer-focused approach and we have successfully built enduring strategies with our clients. When executed, these strategies enable our clients' internal development engines to operate with greater flexibility and efficiency.

As the pioneer of the Embedded Solutions™ model, we have mastered the art of elegant transformation as sponsors realign their operating model to more efficiently manage their development portfolio with greater flexibility and control.



And doing it in an interesting, educational, and interactive way.

The uplifting TV feel is more than just story telling. It's a portal enabling real people (who may be slightly more science literate and proactive than the average Joe) to step into the strange world of genealogy and genomes. And if they click that research-consent box, to get them beyond their family trees, 23andMe will transport its customers to the world of life science research and drug development, resulting in an aggregation of numbers that spell influence, with the potential to impact the healthcare industry in ways entirely unforeseen. The path also leads individuals and their genomes into yet unexplored jungles of personal data privacy, theft through hacking, and the profit motive that drives the big Pharma enterprise.

---

*In addition to connecting people to their ancestral stories, 23andMe is taking a circuitous route to connect them to something far more fundamental: their own health*

---

### Double Deal

23andMe kicked off 2015 with an announcement that let everyone in on what the Mountain View, Calif.-based personal genetics company has been up to since it fell under the scrutiny of the FDA in 2013.

In an interview with *Pharm Exec*, Andrew Page, president, and Emily Drabant Conley, Ph.D., director of business development, stressed that the two disclosures would not be the last this year. In 2014, the company signed 14 deals and anticipates announcing several more this year, though not all will be made public.

The first announcement, on Jan. 6, is reported to earn the company \$10 million upfront and may tack on \$50 million if the company achieves certain milestones with Genentech/Roche. The goal is to identify new therapeutic tar-

gets for Parkinson's disease by generating whole genome sequencing data for roughly 3,000 people in 23andMe's Parkinson's disease community. 23andMe followed up on the Genentech deal by announcing an agreement with Pfizer on Jan. 12. The bulletin was timed for maximum buzz as it came on day one of the JP Morgan Healthcare Conference in San Francisco, a four-day affair known for industry deal brokering.

Though the release regarding the Pfizer pact does not disclose dollars, it promises to be wider reaching than the Genentech deal. 23andMe will allow Pfizer to access its "research platforms, including services and research portal analysis of 23andMe's genotyped population of over 850,000 individuals," according to the announcement. Eighty percent of the individuals in the database

have granted the company research consent, it notes.

In addition to the broad platform agreement, the deal with Pfizer also includes a more narrowly directed mandate to enroll and genotype 5,000 people into a new lupus research community, which will integrate medical records and targeted bio-samples.

The two agreements illustrate just a portion of the spectrum of deals that 23andMe can make, noted Page. The two pacts are different in depth and focus. With Genentech, the collaboration is narrow, centered on Parkinson's disease. The deal with Pfizer provides the company with access to 23andMe's research platform, including services and Research Portal analysis of 23andMe's genotyped population who have consented to participate in research. As a part of the agreement, the companies



Anne Wojcicki

will collaborate on certain genome-wide association studies, surveys, and clinical trial recruitment. One of these collaborations will be a longitudinal study to better understand the genetics of lupus. The remaining unannounced deals inked in 2014 promise to embody a fairly even split of narrow and broad collaborations, Page told *Pharm Exec*.

In addition to working with biotech and pharma companies, Page added that 23andMe's allies will also extend to include academia. The company expects to make several more announcements on these arrangements in 2015. Lastly, one of 23andMe's agreements is with a company in "another category," which should be announced in the near future, he said. Whether this deal is with a contract research organization (CRO,) a device or diagnostics firm, or something else entirely, the conclusion to be drawn is that quarrying 23andMe's heaps of data is emerging as a true source of competitive advantage for big Pharma.

The announcements seem substantial in scale considering 23andMe is still somewhat of an unknown to many in the industry. Its operations and offerings are just beginning to crystalize, and the disruptive potential across healthcare remains theoretical. Given the deals in place—and those yet disclosed—the in-

dustry now sees the 23andMe story as more than a pleasing narrative. Instead, companies are kicking the tires in anticipation of an exhilarating test drive.

### Target: Big Pharma

23andMe has enjoyed the limelight thanks to its A-list founders, Linda Avey, Paul Cusenza, and Anne Wojcicki, the latter now separated from her billionaire Google co-founder husband, Sergey Brin. Its deep-pocketed financial backers include Yuri Milner, Johnson & Johnson Development Corporation, MPM Capital, The Roche Venture Fund, Google Ventures, and New Enterprise Associates. The association with Silicon Valley's financial elite has certainly helped it build credibility as an industry innovator. But since its launch in 2006, many leading opinion makers in healthcare have viewed 23andMe skeptically; questioning how a company pitching a superficial message around genomics linked to genealogy kits could ever impact the healthcare industry.

In fact, forging complex research agreements with biopharma firms has been a major part of the company's strategy all along, explained Drabant Conley. With the support of two biotech and pharma giants, the urge to partner means that 23andMe is positioning to do a lot more than simply sell genetic and genealogy services to the genome-literate mass consumer, at \$99 a pop.

"We've been preaching since inception the impact that learning about genetics can have on the individual and on the research or target discovery process for pharmaceutical and biotech companies," said Page.

The agreements are a "natural outgrowth of what the company has been focusing on since our inception," said Page. "Stepping back, we first focused on engaging and empowering individuals around taking control of their own genome. With this engagement, the database grew, and as it grew we started to have statistically significant epidemiological cohorts of certain rare diseases for pharma and biotech to take an

interest in," he explained. That's when 23andMe's deals started to be formulated. Early collaborations were small and geared to validation work, with the 23andMe's team of PhDs offering products designed to facilitate information exchange, answering questions posed by its customers in a highly collaborative and interactive way.

As the company ramped up its capabilities in 2013, the database became large enough (now with around 850,000 individuals and 80% consented into R&D testing activities) for the firm to categorize and automate its offerings so that it no longer needs any manual consulting service. However, the company

With this information, researchers can search for and potentially identify new associations between genes, diseases, and traits more quickly than through the traditional research model, according to the company.

23andMe opts for diversity in working with collaborators, including academics and scientists, non-profit organizations, and for-profit companies. By partnering with the greater scientific community, 23andMe's goal is to accelerate meaningful discoveries through the accessibility of its data. Building and maintaining this powerful research platform, safeguarding participants' data, and allowing researchers to access this



*"2014 was a great validation year for our database proving the value of genetic and phenotypic data and re-contactability of patient cohorts working in concert. But it's just the beginning."*

—Andrew Page

still does a great deal of custom analysis and consulting. These more automated offerings are what 23andMe are starting to roll out now, Page said.

"What used to take months or years, or may have been impossible, can now be done very quickly with sophisticated searches," he explained. "2014 was a great validation year for our database proving the value of genetic and phenotypic data and re-contactability of patient cohorts working in concert. But it's just the beginning."

23andMe sees "individuals as partners" not study subjects. With some 650,000 partners opting in, the company can apply the largest data set of its kind to any number of platforms. Its research services also include the 23andMe Research Portal, which allows qualified researchers outside of 23andMe to run queries on de-identified, aggregate data.

resource for even more scientific discovery are core to the company's mission.

Even though 80% of 23andMe's database population opted to sign research consent, many may not have grasped the fact that data from their saliva sample might be sold to companies like Pfizer. But the clear consent protocols and evident awareness of 23andMe about real diseases and the suffering, difficult-to-treat patient populations should help pacify those who have been critical of 23andMe on data privacy.

### Not eye to eye: The FDA stir

Up to now, 23andMe's primary headline grabbing moments have highlighted its struggle with the FDA over just what it can tell its customers about themselves. In November 2013, the FDA sent the company a warning letter demanding it stop marketing its saliva collection kit

and genome services, stating it was “intended for use in the diagnosis of disease or other conditions or in the cure, mitigation, treatment, or prevention of disease, or is intended to affect the structure or function of the body”—uses for which 23andMe had not gained marketing clearance or approval.

In its early days, 23andMe strove to be an interpretation platform across a bunch of different modalities, from ancestry to disease carrier status and health risk, explained Jason Bobe, director of the Sharing Lab in the Department of Genetics and Genomic Sciences of Icahn School of Medicine at Mount Sinai in New York. But that goal clashed with the FDA’s authority to regulate genetic tests and health risk reporting.

Both criticism and support rained down on the company. Some have called the company out for its moxie in dealing with the FDA, claiming that the company’s raffish approach to regulatory compliance would be its undoing.

Others support the 23andMe mission to get individuals involved in their own healthcare through greater knowledge of their own genomes. Scores of devotees petitioned for the FDA to allow the personal genomic service, both to encourage people taking action for their own health as well as those with a more libertarian bent that the government should let the firm operate, explained Mike Jones, a senior campaign director at Change.org. Additionally, a commentary in *Nature* in January 2014 called FDA’s crackdown unwarranted with little evidence of damage to patients, stating the agency was “acting on the basis of speculations of potential harm rather than reported harm.”

Following the FDA’s forceful response letter, with which the company quickly complied, 23andMe said it would continue to provide “ancestry-related information and raw genetic data without any interpretation.” 23andMe has continued to work with the agency on refining its test. Resolving the dispute is a top priority, the company states.

This February did offer a harbinger of détente with the agency as the company announced it had been granted authority to market the first direct-to-consumer genetic test under a novel devices regulatory classification. FDA will allow 23andMe to market the Personal Genome Service (PGS) for carrier status testing of Bloom syndrome through *de novo* classification, according to the Feb. 19 release.

Since the FDA put the skids on its personal genomics opt-in plan, 23andMe has struggled to walk a tightrope between public praise for its transparency and concern with the implications of this for protection of individual privacy. A stubbed toe occurred in September last year when Vox.com profiled a less-than-happy ending to a genealogical search conducted through 23and Me. Newly found familial connections might not be the joyous occasion we often envision. Just picture a call or email informing you of different parentage than you previously thought, or maybe your child turned out to have a different birth parent.

The article caused a change in strategy, which had been shifting to “giving people fewer warnings about the secrets they may unlock in their DNA.” Instead, Wojcicki wrote customers that the company had erred, saying, “I do not think it was the right call to promise we would automatically opt-in those customers. Core to our philosophy is giving customers maximum choice and empowerment through data.” The apology also included a promise to be more vigilant by creating a new position of company privacy officer.

23andMe followed through by hiring former federal privacy watchdog Kate Black, with the title of privacy officer and corporate counsel. In case you’re wondering what kind of resume a privacy position might require, Black has extensive experience in the burgeoning field of healthcare IT and privacy. She was employed at the Office of the National Coordinator for HIT, U.S. Dept. of Health and Human Services, and pri-

or to that, as health privacy counsel for the Center for Democracy and Technology in San Francisco.

### Re-contacting & re-consents

“What excites me is the ability to take genomics to the masses. By providing education, the company is essentially providing genomic services attached to a bicycle on training wheels,” quipped Bobe. There is great appetite from the public to be able to access this information at all levels, from people looking at their genomes strictly for genealogy, and, of course, it goes without saying that people want to tailor their backgrounds to obtain better, more effective therapies, said Bobe.

Even if there is a reticence on the part of some genealogy clients to check all boxes “yes”, 23andMe is counting on the majority of its customers to stay interested enough to want to contribute to the expanding database.

In some cases where the company is working with a third party, like the deal with Genentech, 23andMe will go through a re-consent process. When an individual initially purchases and registers a genealogy kit, 23andMe has readied a specific and clear consent document that will establish that the individual agrees to participate in research, Page said.

23andMe is all about transparency to consumers, noted Page. It’s all about consumer choice to participate or not. And they can change their mind at any point in the process. There’s also institutional review board (IRB) oversight, which is essential, added Page. “The integrity with which we conduct research is paramount. Otherwise we don’t believe the research partners contracting with us would be comfortable trusting mission critical research on any relationship with us.”

Most patients are taking the stance of willing to be a small part of a large study, on the understanding that their identifying information won’t be shared without additional consent, Page noted.

For the Parkinson’s study with Ge-

nentech, all participants have consented, and the company will be re-consenting them for this program, he said. This will give 23andMe the opportunity to further engage around this active patient community, looking not just at the information it has, but to ask more questions and add greater detail. We'll also be sequencing whole genomes of roughly 3,000 of that study cohort and doing additional studies on them."

Page is optimistic that patients will largely agree to re-consent even if patients understand that their de-identified data is being shared with a third party pharma company. One reason for publicizing the Genentech deal is that, "we're hoping that the awareness of this study will cause our Parkinson's community to increase in size so that we then have even more participants in this study," said Page. This particular community has been around for a while. But it's unclear what the re-consent rates will be. "We hope it will be healthy, although something up to the 80 or 85% level might be on the high side," he noted.

According to experts interviewed by *Pharm Exec*, 23andMe is now positioned to be successful because patients are looking to be active participants in research. Getting involved and taking action in one's own health, or that of a loved one, is probably one of the "top five trends we are seeing at Change.org," said Jones. Patients want more control and more say over decisions about their own health so they are demanding more information, Jones added.

"Everyone talks about big data and its enormous potential value. But what 23andMe's story shows is that there is also real value in relationships," said Bobe. "You really want both. The ability to have trusting relationships with individual patients, particularly around matters of health and medicine is huge," he said.

There is great value in being able to form a relationship with research participants and analyze them over time, as they go through their lives,

take medications, experience disease and other health changes, rather than just a snapshot of a brief moment in a patient's life. It's akin to the power of social media to connect people, bring together groups, and to maintain trusting relationships over extended periods of time.

### Research acceleration

What has been the interest level from pharma companies in getting involved with 23andMe? Who has approached the 23andMe for collaboration and who has been hesitant? The answers depend on the organizations and their ability to reform

least to see what's on the other side.

Some companies have seen the information assets that 23andMe has developed and have approached the company for that reason. In the case of Genentech, "we've been very publicly committed to Parkinson's and have built the largest Parkinson's community in the world by a significant margin, and they're intrigued by that," said Drabant Conley. Building these assets, as well as work the company has published, has been the impetus to opening many of these partnership doors.

For other companies, as in the context of the collaboration with Pfizer,



*"Rather than just taking data from patients when they come to a clinical center, which is the old method, now we're going to give you data about yourself, about your genetic heritage, and about the study."*

—Emily Drabant Conley

their processes, replied Page. Certain organizations have had breakthroughs using genetics in their own discovery processes, so building on that, a partnership with 23andMe is a natural step.

But 23andMe carries its own ambitious agenda in to these relationships: to be a disruptive force, accelerating innovation into virtually all aspects of drug development. The notion of 23andMe's introducing genetics into the research process is disruptive to the traditionally slow, procedurally dense, toxicity animal-based testing process of drug development, said Page. Given this bold pledge to disrupt and accelerate time to market, as well as the company's commitment to bolstering this with financial and scientific backing, there's little doubt there is interest from big Pharma and the biotech companies to bang down the door, at

they've been interested in developing drugs where there are challenges and limitations based on current methods and tools. They see 23andMe as greatly enabling some things that are very challenging to do in the current, more traditional clinical trial setting.

Clinical trials in some indications might define success by getting a drug to work in 30% of a patient population. But if you could figure out on the back-end before launching a trial covering that 30% and not the other 70%, and thereby target a trial in patients with a specific symptom profile or genetic background, you can really increase your odds of success, explained Drabant Conley.

In the case of lupus, it's really not so much about target discovery, but about patient segmentation and understanding the underlying disease, she agreed.





A Symbol of Excellence

# Innovation

hears a different story

At ICON, innovation means embracing change and finding better ways of working. Our innovation enables our customers to make faster, more informed decisions and to deliver clinical studies better, safer and more cost effectively.

**Contact us today to hear how our innovation is shaping the future in clinical trials;**

**Adaptive Trial Design  
Site Performance  
Patient Centric Monitoring  
Patient Reported Outcomes**

Email: [Enquiries@iconplc.com](mailto:Enquiries@iconplc.com)  
[ICONplc.com](http://ICONplc.com)





Photos: Joseph Schell

# Behind the Boom in Biotech

## Nine Stories of science, salesmanship—and uncertainty

**O**n the margins of January's annual JP Morgan investorfest, *Pharm Exec*, with logistical support from health communications firm Russo Partners, convened a Roundtable discussion with nine company aspirants for leadership in the hard science of small biotech. The dialogue reveals the rigors of building a modern medicines enterprise, where calculated tolerance for risk is the essential nutrient of success. The shared narrative is about the ruthless logic imposed by market demands for differentiated therapies that fill the vacant spaces of patient need, with no place for the come-by-chance serendipity—and easy profits—associated with a broader public image that amounts to warmed-over science fiction.

— William Looney, Editor-in-Chief

*William Looney, Pharm Exec: Biopharmaceuticals are a highly competitive sector, so I'd like to begin by asking each of you to highlight your company's differentiating "pitch" to investors. Can you summarize the business mission, key*

*therapeutic areas, market potential, and strategic/operational objectives across the three-year planning cycle?*

**Prabha Fernandes, Cemptra Pharmaceuticals:** We address a critical unmet medical need, which is the threat of drug-resistant bacteria in the hospital and community settings. Cemptra has two oral antibiotic compounds undergoing Phase III clinical trials: solithromycin (CEM-101) and TAKSTA (CEM-102, sodium fusidate). Solithromycin is demonstrating superior efficacy compared to one of the most potent existing antibiotics, moxifloxacin. TAKSTA is a novel product to combat chronic infections of the bone or joint that requires long-term suppression rather than the standard treatment course of five to 10 days.

The world is in an arms race against microbes. Big Pharma has largely abandoned the antibiotics segment, and all the generic drugs currently in use are less and less effective against infections. Government has entered the picture with incentives to stimulate development of

### Roundtable Participants

**Daniel Cohen**, Chairman and CEO, Pharnext

**Prabha Fernandes**, President and CEO, Cemptra Pharmaceuticals

**Uli Hacksell**, CEO, ACADIA Pharmaceuticals

**Chris Haskell**, Head, US Science Hub, Bayer HealthCare

**Martin McGlynn**, President and CEO, Stem Cells Inc.

**Mark Rothera**, Chief Commercial Officer, PTC Therapeutics

**Mark Rohman**, President, Biocodex North America

**Spiro Rombotis**, President and CEO, Cyclacel Pharmaceuticals

**Randall Schatzman**, President, Alder Biopharmaceuticals

new, more powerful anti-infective drugs, but the economics of pricing continues to shape where research dollars are spent.

Pricing means that most private-sector R&D is still geared toward intravenous drugs administered in acute care settings—Cemptra's model is different. Our take on the patient perspective leads us to conclude that the highest value attaches to drugs that will work best in the community, rather than in the hospital. Patients want to take their medicine in

a familiar place, at home, where there is low risk of complication or additional infection. This accounts for our interest in oral, in addition to intravenous delivery. It underscores our business mission to develop products that will prove equally effective in diverse treatment settings as well as across the patient population, including children and pregnant women. Financially we are strong, having just last week raised about \$150 million from investors; the offer was four-and-a-half times oversubscribed.

**Randall Schatzman, Alder Biopharmaceuticals:** Alder was founded in 2004. Our expertise is using monoclonal antibodies for disease indications where this novel technology platform has not yet played a role—our lead clinical program (MAb ALD403) is a therapy for migraine, but we are also exploring applications in autoimmune and inflammatory conditions.

Migraine is a debilitating condition that afflicts 36 million Americans, who suffer an average of five such headaches per month. Current treatments embody an abortive approach, in which triptan drugs are taken at the onset of pain in the hope that it will not progress.

Our strategy is to develop a safe, long-acting agent that will prevent migraine from initiating altogether. The drug targets a neuropeptide that transmits pain signals into the central nervous system. This appears to be optimal therapy for patients whose symptoms are frequent and recurrent; in our trials to date, of three- to six-month duration, a single intravenous administration of the drug has suppressed the rate of migraines from as many as 14 per month down to zero, in 40% of the patients enrolled. We are confident that our focus on preventive drug technology will be well received by patients and the clinical community. It's going to change the way migraine is treated—a major improvement in the standard of care. The confidence of investors is underscored by a well-received IPO as well as Alder's inclusion in the NASDAQ Biotechnology Index last month.

**Spiro Rombotis, Cyclacel Pharmaceuticals:** Cyclacel's mission is focused on oral therapies that target the various phases of cell cycle control to suppress cancerous tumors, hematological conditions and other serious diseases. We were founded in 1996 by Sir David Lane, a prominent UK researcher best known for his discovery of the p53 gene as a major tumor suppressor master switch. Our operations are split between New Jersey and Scotland, where we enjoy strong support from government and academia.

Cyclacel has two compounds in clinical development, addressing some of the most challenging areas of cancer therapy. Sapacitabine, a cell cycle modulating nucleoside analogue, is being investigated

work for 17 years. Investors often told us this investigational platform had no future. Our contrarian stance on the science behind seliciclib and follow-on molecules has now been validated. But it took a lot of tolerance for risk to get here. The analogy I like to cite is of being that soldier in boot camp where the drill sergeant says not to befriend your fellow GI's—because only half will be around when you get back.

**Mark Rothera, PTC Therapeutics:** I serve as chief commercial officer and have been working to build a global commercialization strategy to bring the first therapy from our pipeline to patients. Our science is based on a deep understanding of RNA biology and is centered



*“Big Pharma has largely abandoned the antibiotics segment, and all the generic drugs currently in use are less and less effective against infections.”*

— Prabha Fernandes, Cempra Pharmaceuticals

for treatment of acute myeloid leukemia in elderly patients over age 70. It is currently in Phase III trial, under a Special Protocol Assessment agreement with the FDA. We are also pursuing indications for sapacitabine in myelodysplastic syndromes, a bone marrow impairment most common among the elderly, as well as chronic lymphocytic leukemia.

The other compound, seliciclib, is a cyclin-dependent kinase (CDK) inhibitor being investigated for lung cancer and nasopharyngeal cancer. CDK inhibitors are an R&D priority for several big Pharmas, including Pfizer, whose drug for metastatic breast cancer, palbociclib, is likely to be the first such product approved by the FDA. (Editor's note: palbociclib, brand name *Ibrance*, received authorization on Feb. 3).

Led by Professor Lane, CDK inhibition has been the focus of Cyclacel's

on development of orally bioavailable therapies that modulate protein expression for patients who are missing an essential protein that can cause a variety of diseases, such as Duchenne muscular dystrophy (DMD), cystic fibrosis, and spinal muscular atrophy.

The last two years have been transformational for PTC, with a successful IPO and follow-on rounds that gives us a stable position for our research and development plans through 2017. We have worldwide rights to our first drug, *Translarna*, which enables production of essential proteins through read-through of a premature stop codon caused by a nonsense mutation in the mRNA. *Translarna* is indicated against DMD, but the science behind it is potentially applicable to approximately 2,000 rare monogenic disorders. Assuming successful read-out of our confirmatory Phase III trial in

DMD, we plan to launch *Translarna* in the US in 2016, following FDA approval; we are currently launching it in Europe and supplying other regions through early access programs.

In addition, we have a confirmatory Phase III clinical trial on a second indication in nonsense mutation cystic fibrosis, are in the process of initiating a proof-of-concept study in MPS I, and plan to initiate a proof-of-concept study for *Translarna* in an additional indication this year. We hope to file a marketing authorization application with the EMA in the second half of this year for *Translarna* in cystic fibrosis based on data from a previous Phase III trial. Finally, something I am very excited about is PTC's pursuit of another RNA platform, which we call alternative splicing, that holds significant promise in treating spinal muscular atrophy through an oral small molecule drug. An ongoing Phase Ia/IIb trial on a drug candidate is underway in partnership with Roche and the SMA Foundation.

**Chris Haskell, Bayer HealthCare:** I manage the Bayer HealthCare US Science Hub, an externally focused R&D partnering and licensing program that the company established in 2011 to leverage the great science here in the Bay Area. Among other projects, I manage our CoLaborator unit, which provides space, equipment, and expertise to a select number of start-up biotechs. We are located in the Mission Bay district, in close proximity to the UCSF academic programs in life sciences, with whom we signed a master agreement on partnering in 2010. The Hub's therapeutic efforts center on oncology, cardiology, hematology, and, increasingly, ophthalmology, due to the *Eylea* macular degeneration franchise we manage with Regeneron.

Like other big Pharma, Bayer understands that good ideas are not found solely in our own labs; we have to be proactive in a scientific community that is increasingly cross-functional and diverse. As scientific discovery expands into new areas, we seek innovators with

the platform technologies best positioned to yield drugs that advance the standard of care. We bring these innovators closer together, engage with them, and sign deals that aren't your traditional one-off option licensing contracts. It's a strategic approach to licensing, in which academic scientists and industry professionals with deep knowledge of various therapeutic areas work on projects with our research staff. Our approach to partnerships is to be flexible and mutually reinforcing, without the pre-conditions that often discourage ideas that turn out to be commercially promising.

**Daniel Cohen, Pharnext:** I am a geneticist who worked on the mapping of the human genome and helped co-found the company, Millennium, now part of Takeda. I founded Pharnext in Paris in 2007; it is very small, privately held, with only 35 full-time employees. We seek to apply our knowledge of the human genome to identify disease molecular networks containing various molecular targets that could lead to drugs for common and rare CNS disorders. This platform we refer to as pleotherapy, which relies on gene mapping to identify the best low dose combinations of medicines to safely fight most any chronic disease.

Our lead drug candidate, PXT-3003, is a three-drug combination indicated for Charcot-Marie-Tooth disease, a rare neurological disease afflicting around 100,000 patients in the US and Europe that causes atrophy of muscles in the arms and limbs, resulting in severe disability. A small 80-patient trial of PXT-3003 has confirmed improvement beyond stabilization; a larger, 300-patient trial of PXT-3003 is planned to confirm these results. We are evaluating sites, choosing the participants, and hope to communicate the results sometime in the next several years.

**Marc Rohman, Biocodex North America:** Biocodex is a family-owned French pharmaceutical company founded in Paris 60 years ago, with operations in 128 countries. I manage the US and

Canada, which has become our largest commercial region. While our worldwide legacy business is in gastroenterology, we've begun to pursue other areas, including neurology and oncology. Our lead product, *Florastor* (saccharomyces boulardii), is the world's top-selling probiotic (available in over 27 different brand names worldwide); *Totect* (dexrazoxane) is an oncology supportive-care product, and the only FDA-approved treatment for anthracycline extravasation.

Biocodex has a distinct marketing model that relies on professional selling and high-touch pharmacy, where we combine digital access technology with extensive private pharmacy services that are regulatory-compliant. Based on this model, Biocodex is now expanding to oncology supportive care, where we believe there are great opportunities in making clinical activities more productive, providing higher patient compliance, and potentially lowering costs of treatment. Our major business development challenge is anonymity, particularly as we seek to expand our portfolio worldwide. Many in the industry remain unaware of Biocodex's underlying strengths: private ownership, zero debt, significant cash reserves, and a strong, well-positioned commercial sales force. We are actively seeking product acquisitions, scientific partnerships, and licensing opportunities.

**Martin McGlynn, Stem Cells Inc.:** I lead a cell therapy company, so our pathway is distinctive from companies in small molecules or biologics. Stem Cells pioneered the discovery and development of a highly purified, expandable population of human neural stem cells derived from human brain tissue. We have created a commercial model that we believe will yield three product-based applications: neuroprotection, neurogenesis, and neuroregeneration. Our clinical/therapeutic targets include vision loss from dry eye age-related macular degeneration (AMD); chronic spinal cord injury; and various myelin-related disor-

ders of the CNS. The novelty of our approach is in relying on the inherent natural properties of the cells, which, when transplanted, are regulated by the host environment. Thus, the same active biological ingredient, i.e., the human neural stem cell, can be directed in different ways to create useful therapies.

To date, we have completed two Phase I trials for rare pediatric neurodegenerative disorders and have recently completed enrollment in Phase I trials in dry AMD and thoracic spinal cord injury. Enrollment is underway for a Phase II trial in cervical spinal cord injury and we will shortly initiate a Phase II trial in dry AMD. There is enormous market potential in dry AMD, which accounts for 90 % of all cases of AMD, and for which, in contrast to wet AMD, there are currently no therapeutic interventions. Finally, a Phase II trial for Pelizaeus-Merzbacher disease, a fatal inherited pediatric myelination disorder, is of great interest to us, but we lack the resources to fund three Phase II trials concurrently.

**Uli Hacksell, ACADIA Pharmaceuticals:** ACADIA is building a CNS specialty franchise on the foundation of our lead drug candidate, *Nuplazid*, which, if approved, will establish a novel pharmacological approach to treating psychosis as well as being the first drug approved in the US for the indication of Parkinson's disease psychosis. About 40% of all Parkinson's patients have this condition, and their only option at present is off-label use of antipsychotic drugs, which carry a black box warning and are associated with increased mortality in elderly patients with dementia. *Nuplazid* selectively blocks the activity of receptor interactions that cause some of the unpleasant and dangerous side effects of broad-spectrum antipsychotics. FDA has given the compound breakthrough status—I believe it is the first time the FDA has accorded such status for an antipsychotic drug. On the basis of this and some encouraging Phase III trial results, we plan to submit a new drug application for *Nuplazid* during the first quarter of

2015; if approved, we intend to commercialize *Nuplazid* for the indication in the US by establishing a specialty sales force. Looking forward, we see *Nuplazid* as a platform for additional development programs in neurology and psychiatry, including Alzheimer's psychosis, where the market is much bigger than for Parkinson's, and which is the focus of another trial we have underway, and for maintenance therapy in schizophrenia, where the need for better compliance is great.

### Worry lists

**Looney:** *The common thread in these brief comments is the challenging nature of the science you are pursuing. The science is big, the ambition is outside, but your companies are small. How do you*

lead but to stick to your own convictions. We certainly faced some skeptics when we pitched the idea of investing in an expensive large-molecule design when the migraine market was being commoditized through generics. Some biologists questioned the logic of using monoclonal antibodies in CNS. Our response emphasized the treatment implications that would follow from our success: we are building a medicine not for patients with that one-off occasional migraine, but for truly desperate patients suffering from 20-plus migraines a month. It's an entirely different space in terms of medical need, one that drives our will to succeed.

**Rothera:** Years ago, when I began working in biotech, I was often refused a hearing by physicians who doubted there



*“It's equally important to create a 'no surprises' reputation with regulators, who frankly confront the same challenges as we do in keeping up with the constant, rapid changes in technology and science.”*

— Martin McGlynn, Stem Cells Inc.

*manage the low points when the problems mount and resources are eclipsed by investor expectations?*

**Rombotis:** Is there a day in any week when we don't face this dilemma? I have worked in the industry for 30 years. Experience has taught me to be a contrarian because, in this business, the risks are always high—but the rewards can be tremendous. And I am not speaking from a purely financial perspective. When a physician tells me our drug pulled a patient back from the brink, and that three years later she is still in remission because of one small pill taken prior to every meal—ell, then, what we have done is actually a big deal. It's well worth those risks.

**Schatzman:** The major pharma companies have a different perception of risk. It is important not to follow their

was any clinical value in antibody-based treatments. Today, this segment is a \$30 billion dollar business. My lesson from this is to be wary of conventional wisdom, and to pursue objectives based on what the science tells you.

### Regulation: Right for science?

**Looney:** *All of you are making big bets on truly novel technologies that require long lead times for validation. Down in the trenches, as you are, is the regulatory process fit for purpose in helping small companies build that validation case and speed time to market?*

**McGlynn:** Some recent FDA reforms could be helpful to our field, including fast-track designation and accelerated approval status based on Phase II data. Both sponsors and the FDA carry an

obligation to make the process work. Companies must focus on educating all stakeholders, including the regulator, about the science and clinical merits of their technology. The stem cell field has a heavy responsibility here. This is the only way to build trust: to show the FDA that your data is strong and verifiable, and when there is a problem you will come forward and 'fess up to it.

It's equally important to create a "no surprises" reputation with regulators, who frankly confront the same challenges as we do in keeping up with the constant, rapid changes, in technology and science. As CEO, I prefer a "no hype" stance where we simply let the data speak for itself. I can't say this works for everyone—Stem Cells may be paying a price in the marketplace, with a lower valuation among investors than might be the case were we to try to oversell ourselves.

**Hacksell:** Small companies have to differentiate their value proposition to a degree that the major players do not. Positive investor sentiment is critical to our growth prospects. You must spend time with them, creating a strong narrative where you tell them what you have done and what you are going to do, and do it repeatedly until you obtain their trust. I usually meet with potential investors as many as 10 times until I can be certain they understand our story. If you nurture the relationship and keep things honest, there is the opportunity to get them on board.

**McGlynn:** Further to Chris Haskell's point, investors and companies alike have not yet realized the potential for positive impact deriving from the initiatives of regulators to improve incentives for medicines for small populations or diseases for which there is no therapy. Literally, the old clinical trial nomenclatures attached to Phase I to Phase II to Phase III are fading. There are numerous examples where one single trial has been sufficient to obtain FDA approval. It means that the regulator is demonstrating a much higher degree of flexibility. A successful innovator will study that trend

very carefully and adjust its strategy for approval. But it's even more important today to think beyond the regulatory proposition to the value proposition in the marketplace. Cell therapy companies have stumbled repeatedly here, either due to cost of goods or manufacturing and reimbursement issues.

**Rothera:** Investing in clinical trials can be a high-stakes game. Often a company enters uncharted territory in determining which endpoint to select for regulatory approval while also striving to meet the needs of payers. European regulators are pilot-testing a concept called adaptive licensing, designed to bring down the cost of rare disease medicines by focusing on very small populations where the drug is expected to have the greatest benefit, and focusing on analyzing the risk/benefit ratio to speed access to the therapy for patients with high unmet need—in return for a lower introductory price. The catch is that the licensing decision can be revisited over time, in the light of evidence from real world use of the product. This could require additional investments in observational studies or follow-up trials, as well as the possibility that the therapy's initial indication could be changed or even withdrawn. Clearly, for this program to work, there must be a high level of trust and cooperation between regulators, the company, and, in particular, the reimbursement authorities.

**Rombotis:** I wonder about the feasibility of combining the scientific complexity of licensing approval with the economics of drug pricing. Industry lacks the evidence and information base to anticipate the expectations of the other stakeholders. Payers and registration bodies have different grounds for decision-making, which may—or may not—be based on legislative authority. From a purely political point of view, industry also has to recognize that payers operate within fixed global budgets for medicine. That single fact can trump what registration authorities might see in the clinical dossier.

Every other industry except our own is pursuing a market access strategy based on the principle of satisfaction guaranteed—or your money back. It's time to proactively shape the process instead of waiting for change like ostriches, heads in the sand. This means presenting a clear, value-based proposition to regulators and payers, and managing clinical development to avoid front loading all the risks and costs to Phase III, which virtually guarantees your offer price is going to be unaffordable for many constituencies.

### Top of mind: Pricing, access, and IP

**Looney:** *Let's move to P&R issues. How well is payer activism translating to more options for patients, at affordable prices?*

**Fernandes:** Government is supporting some of the development costs of new antibiotics but this is countered by a misaligned approach to reimbursement. Volume sales potential is a key driver of how an anti-infective medicine developed in the private sector gets priced. Public actions like out-of-pocket tiering of reimbursement means that physicians are not empowered to choose the best antibiotic for the patient's condition. You have to try all the weaker drugs first before the patient is really ill and one of the newer, more potent drugs is authorized. I understand the argument about demonstrating value, but restricting access to the full clinical armamentarium can raise the overall cost of treating a run-away infection.

**Rothera:** This trend is driving much of the effort to personalize medicine, by targeting small patient populations for drugs whose benefits can more easily be demonstrated using available data. Everyone talks about a "global price." The reality is that not only are geographic markets very different, with variable delivery systems and levels of affordability, but so, too, are the product/therapeutic segments—these are being sliced and diced, according to genotypes or other measures of clinical differentiation, to a level not seen before. Technology now allows that.

**Schatzman:** Small companies are highly vulnerable to industry-wide pressures on pricing. All it takes is one letter from a senator complaining about prices for one product or a therapeutic category to raise questions about the sustainability of our entire business model. Some categories have seen price adjustments in the double digits due to concerns raised in Congress. In practical terms, that means you cannot raise the money you need, which over time affects the pace of innovation in the industry at large. It's counterproductive.

**Looney:** *What can be done to build a more positive consensus on the link between pricing and support for innovation? Sick fund payers in Germany are suggesting companies could voluntarily furnish more data on the R&D costs they incur in development as a way to justify a premium on price. This concept is embedded in the US ACA reform law for insurers, who must certify that 85% of their revenues are earmarked to support patient claims. Might this provide a possible way forward?*

**Fernandes:** As a small company with an expensive development program for an oral antibiotic, where there are few other alternatives, I know we are going to be pushed on the link between cost and price. We are keeping detailed records of these costs. Our costs are significantly higher today, precisely because of the priority that government attaches to our progress. The FDA, for example, is constantly requesting more information and data to support our dossier submissions.

**Rothera:** Asking industry to account for its R&D costs on a per-product basis raises some questions. Is there an agreed methodology to address the unallocated costs of "dry holes?" Do we add the funding of the extensive post-marketing study commitments that are increasingly made contingent on the grant of the registration license? What about the infrastructure that we must build to actually bring the new drug to patients, a cost which weighs most heavily on smaller companies?

**Haskell:** The main cost driver can never be allocated: it's the nine companies that failed to bring a compound to market, for every one that succeeded. Drug development is expensive because there is no consolation prize—the failures become part of our official P&L statement and are thus borne entirely by the company investing in them.

**Rombotis:** Little is said about the cost of exploratory research not tied to the development of a particular compound. Yet this is critical to advancing medicines innovation overall. In addition, the stakeholder interests outside the drug industry are a major source of

**Cohen:** There is a disconnect between the price of a drug and its cost. Most people are unable to distinguish between these two terms. In particular, there is a lack of understanding as to how development time cycles and failures in our industry raise the cost of a new drug. One of the unstated arguments in favor of innovation is that innovation, viewed over the long-term, actually contributes to lower costs where it really counts, which is in the health outcomes experienced by patients.

**Looney:** *Is IP still a mission-critical function in biotech today?*



*"It's time to proactively shape the process instead of waiting for change like ostriches, heads in the sand."*

—Spiro Rombotis, Cyclacel Pharmaceuticals

inefficiency. We rarely hear about the pricing distortions in oncology, whereby physicians benefit from an add-on percentage to the ex-factory price of an injectable chemotherapy drug because it is administered in-office, as opposed to being dispensed in pill form. I suggest our industry might be better off in working to remove these distortions through the commercial process rather than trying to educate a politician in a debate that will die out because there is no substance to the interchange, only rhetoric.

**McGlynn:** Another item is why perceptions of what our industry does are so different than other industries facing similar circumstances. The perfect analogy is the oil and gas trade, where companies drill 10 dry wells for every gusher. That's the same for us, but we face an added social and political challenge because biopharma investments are expected to accomplish something fundamental: to improve the health of humanity and be accessible to all.

**Fernandes:** There is no doubt as to its importance, especially in my business, where US law offers developers of new antibiotics an additional five years of patent protection. This has had a measurable impact on the pace of innovation in our field. Actually, the patent system can contribute to the quest for better access at affordable prices, for all medicines. Innovative approaches like extending the patent term for therapies already on the market in return for investing in an area of unmet medical need is one way to accomplish that goal.

**McGlynn:** An interesting dilemma has emerged between an approach that focuses on protecting know-how as a trade secret and the traditional strategy of filing a patent, which of course requires disclosure of the innovative step behind an invention. Some of the technology being pursued now will take more than the life of the patent to get approved, much less commercialized. This is certainly true in stem cells, where the perceived value of patents is declining. On top of



# PharmExec.com



## THE LATEST

- News
- Analysis
- Webcasts
- Whitepapers
- e-books
- And more

**www.PharmExec.com** features easy-to-use navigation with content available by targeted category, keyword search, or by issue. Fresh content supplied by *Pharmaceutical Executive's* expert staff as well as external sources make **PharmExec.com** the source for comprehensive information and essential insight.

**Pharmaceutical  
Executive**



# Registration is open

## 2015 Healthcare Businesswomen's Association Woman of the Year event

Honoring:

**Nearly 100 Rising Stars and Luminaries**

**STAR**

Carol Wells, senior director of commercial training and development, Genentech

**Honorable Mentor**

Stuart Sowder, PharmD, JD, vice president external communications,  
Pfizer

**2015 Woman of the Year**

**Denice Torres, president of McNeil Consumer Healthcare,  
a Johnson & Johnson company**



## Register now!

**Early-Bird Deadline: April 2, 2015**  
[www.hbanet.org/woman-of-the-year](http://www.hbanet.org/woman-of-the-year)



Sponsorship opportunities are still available

**May 14, 2015**  
**New York Hilton Midtown**



## BioScience Bounty: Africa's Emerging Geography of Ideas

**Drug and diagnostic start-ups and R&D leaders from South Africa converge in New York to share their boundary-breaking pursuits for therapeutic diversity and “frugal” healthcare innovation back home**

By William Looney, Editor-in-Chief

A few months ago, *Pharm Exec* met with Pfizer to test a simple thesis: does great science transcend the boundaries imposed by geography, economics, medical practice, and culture? The target for this exploration of innovative potential was a delegation of 10 start-up entrepreneurs and R&D

“incubator” executives from South Africa that the company hosted in New York for an interactive series of pitch presentations, live webcasts, receptions, and workshops. The group met with venture capitalists, government officials, academics, and multilateral donors, all experts in the art of business

development. Pfizer gave *Pharm Exec* full access to this week-long exchange of ideas, with the aim to identify actions to break the logjams around great science through a sustainable network of private-sector bioscience enterprises—particularly in emerging market countries like South Africa, where useful precedents exist on delivering health innovations at lower cost to society.

What we learned from our contacts with these risk takers is this: the science is strong, the business strategy is sound, but the competition is fierce—and financing to complete that final, crucial step toward a waiting market is in short supply. Overcoming it depends on succinct messaging around a differentiating value proposition that can be understood by customers; good governance, including improvements in the infrastructure of dealmaking; and cross-sectoral networks to facilitate active partnerships. The stakes are high, because growth of a knowledge-based

service economy is critical to offset the effects of falling commodity revenues. It is also essential to creating opportunity for new entrants to the labor force: Africa, where the median age is 26, has the world's youngest population; you might call this distinctive demographic marker the region's "human energy advantage."

Tackling these long-term challenges is behind a partnership agreed to last year between Pfizer and The Innovation Hub, an incubator group established by the Gauteng provincial government to promote a culture of innovation. Among other activities, the Hub sponsors innovation competitions for local researchers and entrepreneurs through the Gauteng Accelerator Program (GAP) in biosciences. Gauteng accounts for roughly a third of South Africa's GDP and is the location for most companies active in pharmaceuticals and biotechnology. Through the GAP Bioscience Competition, the Innovation Hub works to build an enabling regulatory environment for innovation; ensure that local entrepreneurs in biosciences obtain access to seed funding and relevant technologies; and raise expertise through skills development and training, through such means as a sponsored exchange program in management offered through Emory University in Atlanta.

### Rooting out the best

Innovation Hub CEO McLean Sibanda, who helped select, organized, and led the entrepreneur delegation to New York, summarizes his group's role as an on-the-ground facilitator. "Our business hinges on supporting the growth of small local enterprises in the continent's most advanced and promising location for biopharmaceutical innovation. We develop skills, foster the transfer of knowledge, and provide the intellectual and physical space to enable private-sector innovators to create."

Sibanda told *Pharm Exec* the Pfizer South African entrepreneur exchange program is the first such project under the new partnership,

which also includes discussions on locating some Pfizer R&D work at the Gauteng provincial government's new BioPark research lab and office facility in Pretoria.

The Innovation Hub also worked closely with the African Innovation Foundation, a Zurich-based private group committed to mobilizing innovation across Africa, to select the five South African companies invited to the week-long visit with foreign investors

information on the required steps to achieve full commercialization—like all start-ups, the ventures, while fully validated from a clinical proof-of-concept perspective, remain a work in progress on the ultimate path to the patient.

### Crossing the blood barrier

Medical Assistive Technologies (MAT – [www.MedAssisTech.com](http://www.MedAssisTech.com)) is developing a unique precision engineering tool to

---

*Africa, where the median age is 26, has the world's youngest population; you might call this distinctive demographic marker the region's "human energy advantage"*

---

in New York. According to Foundation representative Pauline Mujawamariya, who directs the group's flagship program, the annual Innovation Prize for Africa, it works to recognize the best innovations in various sectors that (1) represents a major medical advance; and (2) increases health efficiency or lowers costs, all in an Africa-wide context—though appeal to the global market beyond Africa is a plus too. Of the five companies, several have either won the award or been selected as finalists.

"What we saw in this group of private-sector leaders is a finely tuned portrait of the sheer therapeutic diversity and medical relevance of biopharmaceutical innovation in South Africa today," says Pfizer Assistant General Counsel Angela Wasunna, who represented the company on this project. "Much of the group's work is focused on diagnostics, which is a key driver of efficiency in health and is critical to producing better outcomes for patients as integrated health systems on the continent begin to emerge."

The following provides a capsule summary of the business model and products being pursued by each of the five sponsored companies, along with

address a key barrier to safe and effective medical diagnosis and treatment: needle phobia among patients, reinforced by *ad hoc* and often ineffective health worker interventions. Every day, some 123,000 patients in South Africa, and 2.7 million in the US, have a needle inserted to draw blood from a vein. This work is done by staff with varying levels of experience, for patients with a diversity of symptoms, which can make this seemingly simple task arduous—clinical literature reports that nearly a third of these patients require repeated punctures that can spread contagion and lead to hematomas and other potentially dangerous complications.

Like many entrepreneurial ventures, opportunity was revealed through a lived experience: Dean Sher, MAT's founder and CEO, suffered an accident while he was a bio-engineering student at the University of Witwatersrand in Johannesburg; a stray needle puncture left his arm paralyzed for more than a month, further delaying his recovery.

The MAT product solution is an assisted injection device it calls *Vein-AID*, which, when strapped on a patient's arm, uses remote sensing 3D



## Pfizer's Diversity Dividend

*"Pfizer believes that a diverse workforce helps create the socioeconomic transformations so critical to building local, home-grown approaches to health innovation. As part of this commitment, Pfizer South Africa has established an employee forum (PESCO), which focuses on skills development and employment equity. And currently, 68% of Pfizer South Africa's employees are female; each and every one is highly regarded for their skills and contributions to the business."*

— Jennifer Power, Country Manager, Pfizer South Africa

mapping to locate a precise location in a healthy vein and then guides the needle directly to the vein's center. "We saw this technology as filling a major unmet health need, by making what is perhaps the most basic of medical procedures safer and more predictable. But what we really had to do was ensure the device could be available at an affordable price," Sher tells *Pharm Exec*.

That goal was achieved by applying the project team's expertise in IT, biomedical engineering, and materials management to create a scalable software package that brought the introductory price of a *VeinAID* unit down to the \$2,000 range. "We also developed data to show that, with use of *VeinAID*, the time it takes to successfully insert a patient is four times faster," says Sher. "In South Africa, the device will pay for itself in only a few months; there is the added benefit of using fewer needles, shortening the patient queue, and freeing nursing staff to perform other tasks." Sher notes that human intervention is still required to apply the device, so it is not an employment threat to health workers.

MAT's business plan indicates a potential global market of \$4 billion for assisted injection devices, with a sales target of 54,000 *VeinAID* devices in

South Africa alone. What MAT needs most right now is private-sector partner funding—in the \$2 million range—to complete final development of the product over the next 12 months, followed by another year to obtain regulatory approval, beginning in South Africa. "Our goal is to have *VeinAID* up and selling here by the end of 2016, if not sooner," Sher confirmed.

### Better test for TB

**MARTI TB Diagnostics** is the developer of a targeted, cost-effective approach to early detection of tuberculosis (TB), a global scourge which has endemic status in South Africa and kills an average 300,000 to 400,000 patients per year, many of whom are also victims of AIDS. Despite its high mortality rate—globally, the annual death toll from TB exceeds 1.5 million—the disease is curable if treatment is administered early.

MARTI TBD is aiming for that sweet spot, building on the common knowledge that current diagnostic tests are slow, cumbersome, and even hazardous because of the reliance on sputum samples from the lung, which increases the risk of infecting health-care workers and other patients during and after the procedure. There is also the fact that sputum-based

diagnostics fare poorly in detecting the TB bacillus outside the lung, even though about one of every five cases of TB originates outside the lungs, in the brain, or the joints. Even the most modern diagnostics require at least an overnight to post results, while the method still regarded as the "gold standard" takes weeks. This raises issues around motivating test subjects to return for their diagnosis and minimizing the possibility of an infected person transmitting the disease to others during the wait period.

"Our company was established to create an alternative to this unsatisfactory situation, where the limitations of current diagnostics technology perpetuates the spread of a highly contagious disease," says Gerrie Mostert, principal business adviser to MARTI TBD and IP commercialization officer at the University of Pretoria, which currently holds 100% of the company's issued shares. What MARTI TBD has done is develop an entirely new blood chemistry process that screens for a specific antibody known to be present in all patients with active TB. The test requires extraction of only a single drop of blood and is conducted using existing low-cost, off-the-shelf instrumentation, directly at the point of care, with results available to physicians and

patients in less than one hour. "It's mobile, portable, simple to administer, and accurate, especially for patients that co-present with HIV," Mostert notes. He says the test is being validated in line with World Health Organization (WHO) rules on the efficacy of TB diagnostic tools. The company is confident its blood-derived methodology will lead to significant savings for health facilities on the front line in managing the disease.

The next step for MARTI TBD is to conduct a clinical trial testing its approach in conformity with WHO guidelines, the results of which the company hopes will produce a WHO endorsement sufficient to persuade South Africa and other countries to subsidize the diagnostic in the public health setting. According to Mostert, "reimbursement for TB diagnostics is well established today in most countries with a high incidence of TB. Our value proposition will be reinforced if we enter the market on the basis of a supportive policy directive from the WHO."

Mostert and MARTI TBD operations head Carl Baumeister are now seeking potential foreign partners to help conduct and fund the clinical trial. To appeal to investors, MARTI TBD has secured patents on the technology, granted in the US and dating back to 2007, while a number of more recent patent applications are pending in several countries. The IP is registered to the University of Pretoria, which will increase the technology's accessibility within the South African public health system, but MARTI TBD has an exclusive worldwide license to the IP for commercialization purposes. Baumeister estimates an initial market amounting to \$150 million annually in South Africa alone, based on the more than seven million diagnostic TB tests conducted within the country each year. Globally, the market could reach as much as \$1 billion if the technol-

ogy catches on with regulators and healthcare workers.

### The eye tells

**Medical Diagnostech** ([www.meditech.co.za](http://www.meditech.co.za)) is pursuing a business model of low-cost solutions to help the public and private sectors test for health conditions with a high social or public safety impact (i.e., fertility/pregnancy, sexually transmitted diseases, HIV, malaria, and drug/alcohol use). According to company founder Ashley Uys, Medical Diagnostech got its start in 2006 after he recognized how changing social and economic conditions in South Africa were forcing government and the private sector to step up their game in addressing the relationships between dependency and impairment, aberrant behavior, petty crime, declining productivity, and higher healthcare expenditures. That insight has proven its worth — the company is now profitable, has 55 employees and owns a state-of-the-art manufacturing facility near its base in Cape Town.

The company benefited from the early support of the government's Industrial Development Corporation, which provided a \$160,000 grant to help push Uys' pitch, citing the major business opportunity in locally developed diagnostics suited to the most prevalent public health challenges. Medical Diagnostech's first product was a test kit on methamphetamine abuse, launched as a distribution deal on a kit imported from the US. That led to Uys winning the South African Breweries Innovation Kick-Start Award, an annual national competition on small-enterprise development; this was followed by another government grant for lab space to conduct research on additional home-grown solutions to fill the gap in the testing device market.

Uys decided on two criteria to plug the testing gap: quality and sensitivity. "We could not compete against the rep-

utation of US and European producers if we failed to match them on quality, while sensitivity is a live or die condition for any test, because of the adverse consequences that occur when you get a readout that is false positive." What the company came up with is a new image capture technology, *OculusID*, which relies on reaction time in the eye pupil—which is very sensitive to light—to detect levels of impairment when an individual is exposed to alcohol or a drug or narcotic. The rate of change in dilation or constriction of the pupil can be calculated and then compared against predefined benchmarks to determine whether such impairment exceeds legal boundaries, a process that is both clinically accurate and verifiable.

"It's a non-invasive, hygienically safe alternative to breath, blood, or saliva testing; *OculusID* is also unique in that it can be applied to both eyes at the same time, in contrast to only one eye in imported competitor products." Uys notes that application of *OculusID* to drug or narcotic impairment represents a significant saving for employers because it eliminates the need for random work force testing.

*OculusID* also has potential in the diagnosis of chronic diseases like diabetes and hypertension. Why? According to Uys, *OculusID* is a portable and simple technology, making it ideal for home use by patients in tracking their blood pressure or blood sugar. And it comes without the discomfort of conventional, invasive, cumbersome, and often inconclusive testing devices.

To fully commercialize *OculusID*, Uys says his company has to complete three steps. First is conducting a randomized, evidence-based study to document the product's quality and accuracy. The second is finding a foreign partner to share this and other development costs in return for rights to the product outside Africa; Medical Diagnostech plans to retain rights on the continent. Third, Uys wants to secure

## Start-Up Saga: Bridging Africa's 'Valley of Death'

South Africa may have the most developed and sophisticated market for biopharmaceuticals on the African continent, but discussions with executives of the five start-ups participating in the Gauteng Innovation Hub's recent visit to New York reveal a few critical shortcomings. First and foremost is the absence of a strong network for private-sector investment capital. The existing infrastructure of commercial banks along with that regional powerhouse, the Johannesburg Stock Exchange, is geared almost exclusively to extractive industries. Private venture capital firms are rare, without a supportive regulatory backstop to compensate for risk. And while there is a disproportionate number of wealthy angel investors compared to the rest of Africa, this group remains largely ignorant of the life sciences sector. "Generally speaking, private funders want a return on their investment in three years or less—far too short for the long development cycles in biotech," says Nuno Pires of Altis Biologics.

Government seed grants, which are still the principal support for South African life science start-ups, often lead to a perverse result: these provide just enough money to help companies demonstrate their product concept is feasible, only to have the entire effort collapse when no private investors come forward to underwrite the larger outlays for end-stage clinical trials and other regulatory benchmarks required to obtain a final market approval. Adds Pires, "from an economic standpoint, it is an inefficient allocation of resources; even assuming you do eventually get the necessary private financing, the most realistic outcome is that the innovator, whose idea takes wing with a government grant, ends up holding a minority position in his own company."

Still, he sees a silver lining here. "Not having the funding concentrates the mind very nicely. It forces you to focus on what is essential while keeping things simple, with solutions that are practical and scalable in line with the resources in hand." Pires tells *Pharm Exec* that his company has spent only \$2 million so far to get its novel bone regeneration therapy to market, while his US competitors have dropped more than \$600 million in pursuit of the same objective, albeit around a larger geographic map. Could there be a future in the science of the scarce?

Government can play a direct role in addressing a second barrier to commercializing good science in Africa. This is the lack of institutional interconnectivity among the national innovation ecosystems of the continent, a situation that exists even within South Africa itself in the form of competing regulatory objectives, poorly prioritized trade relationships, and the misaligned incentives that drive practices in industry, academia, and government. Most of the entrepreneurs contend public policy can change this dynamic, especially in making the transfer of technologies from lab to the market more efficient via stronger IP protection and subsidized incentives geared toward small enterprise. Rather than a welter of underfunded initiatives driven by politics and administered inconsistently, governments should concentrate on a few things, done well, with an eye to making local African expertise more competitive internationally.

— William Looney

private financing for a planned antibody production and evaluation facility in South Africa to, among other things, ensure the company maintains control of the data compiled through testing. "There is a huge add-on potential in selling our aggregated data stores to other vendors, inside and outside South

Africa," says Uys. "The challenge is we don't yet have the capability to take this to full marketing by ourselves."

### Targeting binge behavior

**Swazz Innovations** is taking the impairment concept to the retail level, with what it contends is the world's

first OTC product to counter the dehydration that creates the post-party hangover. Called a hangover shooter, the product carries the brand name *Lobocla*, which is alcohol spelled backward. The company established to develop this idea was founded by 26-year-old serial entrepreneur Llewellyn Tshesane. "My skill is not in science but in mobilizing people behind ideas that can make a difference both in the marketplace and society at large," Tshesane tells *Pharm Exec*. "The innovative thrust behind *Lo-*

*While [in South Africa] there is a disproportionate number of wealthy angel investors compared to the rest of Africa, this group remains largely ignorant of the life sciences sector*

*hocla* is that we have engineered it as a pharmacological product but without the hurdle of regulatory approval or pharmacy-only requirements on distribution, which limits access." The medical need is unquestioned, as WHO statistics indicate that, in South Africa alone, per capita consumption of alcohol is about 8.2 liters per year.

*Lobocla* combines a number of natural ingredients that help the body retain water and aids in the breakdown of acetic acid in the liver while a person is impaired. The company, with support from the Gauteng Innovation Hub, has conducted human tests to prove the shooter, when distilled in water, is non-toxic. "It is safe and can be consumed like Coca-Cola. As a shooter, its packaging takes up little space on the store

shelf—that appeals to retailers.”

The Swazz team conducted an independent clinical trial of *Lobocla* in November, which proved the product was efficacious in improving coordination and motor skills. What the company needs now, says Tshesane, is outside manufacturing and distribution capabilities to scale up for production of the product in South Africa and also expand into other countries in the region and eventually to the US and Europe. “We require roughly \$5 million dollars to purchase manufacturing equipment and establish a national network of retail distributors,” he revealed. “Our aim is to create an entirely new industry and to do that we have to hit hard and fast.”

Tshesane says *Lobocla* will be ready for country-wide rollout by mid-year.

### Biologic for bone repair

The final participant in the investor visit was **Altis Biologics** ([www.altisbiologics.com](http://www.altisbiologics.com)), which is focused on regenerative medicine products derived from biologic materials, with a therapeutic specialization in skeletal and dental orthopedics. The company’s two founders, Dr. Nicolaas Duneas and Nuno Pires, took an early interest in scientific research on the bone morphogenetic protein, which has been shown to facilitate bone growth in patients with traumas of the spine, hip, and craniofacial bones. Duneas’ work on the ways that natural materials can generate new bone led to the development of a custom, porcine tissue-derived injectable formulation—Osteogenic Bone Matrix (OBM)—that can be grafted in humans to spur bone regeneration and growth in these patients.

The novelty and clinical promise of the OBM technology was formally recognized last May, when it was awarded the 2014 Innovation Prize for Africa given annually by the non-

profit Africa Innovation Foundation for new products that spur Africa-led growth and development.

“OBM is a significant, high-yield advance for the auto graft replacement industry. It carries major innovative potential to surgeons and patients, because with OBM there is less need for additional invasive procedures geared to harvesting replacement bone from a donor site, elsewhere on the body,” says Duneas. He notes that approximately 10% of all fractures are non-unions, which can be severely debilitating and requires some form of artificial bridge or fusion. Altis OBM is indicated to treat every one of these indications, with especially good results in spinal fusion, a surgical procedure conducted some 700,000 times a year in the US alone.” Duneas contends the global market for regenerating bone biologics is now worth about \$1.6 billion annually.

Mindful of the international market potential, Altis OBM has been patented in the US and six European countries and there are additional manufacturing know-how and trade secret protections. Because initial funding for clinical studies was secured through the South African government, it was important that the company has been able to position OBM at a price that is about a third of that offered by competitors based in the US and Europe—a distinct competitive advantage in the African context, where healthcare funding is limited.

Altis OBM has been authorized by the Medicines Control Council for limited trial use among selected surgeons in Johannesburg and Pretoria. Some 350 patients have been treated to date. According to co-founder Nuno Pires, who handles business development for Altis, financing is being sought from the private sector for two projects: (1) further clinical trial work to win final regulatory approval for Altis OBM as a Class III

medical device; and (2) building a world-class manufacturing capability. The company is also interested in negotiating licensing deals for markets outside South Africa. Pires says the company is working closely with local orthopedic surgeons on how Altis OBM can raise the bar on surgical techniques that improve outcomes for patients. “Our strategy is to address innovation not solely from a pharmacological perspective but from an engineering orientation too—to help make orthopedic surgery safer and less invasive, with fewer side-effects for patients.”

A big step in that direction was the company’s recent success in winning reimbursement approval for Altis OBM from one of South Africa’s largest private-sector health insurer, Discovery Health. “Attaining that was critical to our objective of getting this technology directly into the hands of surgeons who treat patients, and can directly observe results with our technology,” Pires tells *Pharm Exec*.

### Pfizer's next round

The contacts and insights derived from this first visit have persuaded Pfizer to continue its outreach to innovators, not only in South Africa but in the rest of the continent as well. A second, reverse visit involving US-based investors and business development experts to South Africa is under consideration. Beyond that, the South African group will use what it learned from the time spent in New York to put together customized plans to hone their investor pitch, and broaden their marketing strategies to attract VC and licensing partners from outside the region. Each company now knows that technology is now global, and to maximize any commercial opportunity they must follow the science—wherever it leads. **PE**

William Looney is *Pharm Exec*’s Editor-in-Chief. He can be reached at [wlooney@advanstar.com](mailto:wlooney@advanstar.com).

# Tomorrow's Selling Strategies: Invest & Test

**A new PwC survey reveals a strong need to fully embrace alternative commercial methods—not in a blind frenzy but through careful evaluation of which new sales strategies have true innovative potential**

By Rick Edmunds, Rolf Fricker, Stephan Danner, and Nelia Padilla, PwC

**F**aced with a changing commercial marketplace, pharmaceutical leaders are investing heavily in new but largely unproven selling strategies—and they will continue doing so even though many of these new approaches are producing mixed results. That's the key finding from PwC's 2014 Strategy & Global Pharma Marketing & Sales Study, which surveyed more than 150 senior sales, marketing, and strategy executives to find how pharma companies are wrestling with a marketplace in flux.

Much of the uncertainty stems from the decline of the in-person sales rep model that has been the industry's standard approach for decades. And while there have been clear successes in making evolutionary improvements to that model—such as the increased use of key account managers (KAMs)—pharma executives intend to devote more resources to novel methods of reaching customers, despite the fact that success metrics for those methods remain largely undefined.

In placing their bets on new techniques with transformative potential, companies must recognize that the efforts to date have not adequately taken into account customer needs. At the same time, too little attention has been paid to establishing effective metrics to assess their validity and identify best practices. In short, before throwing good money after bad, the pharma-

ceutical industry needs a more basic understanding of its strategic objective and of how it can use new practices aligned to a measurable return on investment. Industry leaders must also become more outcomes oriented and overcome stubborn organizational and operational barriers that slow the pace of innovation in interacting effectively with a new breed of customer.

## **New take on an old model**

In previous years, our surveys focused on the future—for example, asking pharma executives where they planned to invest. While a number of predictions can be drawn from this year's survey, it primarily paints a picture of where the industry and its players stand at the moment. Where are they finding success and how are they building the right strategy or metric by which to accurately assess that strategy? Our goal was to scrutinize conventional wisdom, and to separate some of the industry's myths from its realities.

The results confirm that the industry is awash in new sales and marketing strategies and tactics, at various stages of implementation. This is no surprise—traditional tactics have often become cost prohibitive, the commercial marketplace has much greater complexity, and new regulations have restricted historical options, throwing the points of contact between pharmaceutical companies, their sales

teams, and the providers themselves into a state of protracted turmoil. With the traditional landscape under pressure, many companies have shied away from unproven new commercial models (NCMs), relying instead on tweaking their old models to adapt to a changing market.

Nearly three-quarters of survey respondents agree that pharma companies have mainly repurposed the sales-rep model toward account management rather than creating any truly game-changing innovations. This reliance on tried and true models is understandable. These innovations, while incremental, clearly represent progress toward a better overall strategy moving forward.

But much work remains to be done. As one of our respondents pointed out, “sales reps will continue to be important but companies need to review capabilities to provide what is essential in adding value to customers.”

## **New channels, new content**

A discussion of the new commercial model begins with a look at these new channels and content strategies.

The new channels being tested include:

- » **KAMs:** Servicing key institutional providers through a single account manager.
- » **Clinical sales forces:** Sales reps with substantial medical knowledge of specific disease areas and relevant therapies responsible for clearly communicating the benefits and uses of specific therapies to health-care providers (HCPs) and other key stakeholders.
- » **Patient/physician portals:** Web portals and other technologies that patients, physicians, and other stakeholders can access to obtain information about diseases, treatments, products, or services.
- » **Social media promotion:** Using social-media sites (Facebook, Twitter,

etc.) to engage consumers and provide medical information.

- » **Dynamic channel management:** Integrating content and engagement history across HCP-directed channels to create a seamless experience.
- » **Digital tools:** Digital applications used to engage HCPs, provide disease, product or service-specific medical education, or detail HCPs for product or service promotion. New content being taken to customers includes:
- » **Product budget/outcomes models:** Health economics and outcomes research that can be used to assess the impact of a therapy on relevant patient groups.
- » **TA or product-specific value-added services:** Going “beyond-the-pill” to offer services targeting patients, HCPs, or other key stakeholders aimed at improving treatment outcomes or patient experiences.
- » **Above-the-brand services:** Ancillary support services done “without-the-pill” (e.g., improved diagnostics, home monitoring) targeting HCPs and other care providers focused on improving the quality of care.
- » **Joint research/analytics with both payers and provider institutions:** Collaborative research and analysis providing real-world evidence to help determine how products and services can either be used to improve the efficiency and efficacy of care or integrated into approaches to improve care outcomes.
- » **Disease management services:** Services that improve management for chronic conditions by enabling coordination and communication between patients, care-givers, HCPs, and other key stakeholders.
- » **Innovative pricing and contract structures:** Agreements that value therapies based on standards, established in collaboration with payers or providers, that go beyond those traditionally used to value therapies. It’s crucial to remember that, as the

market becomes more contested, regulated and complex, with more stakeholders to serve, adaptation to this new environment is far from simple. Executives are tasked with reshaping their businesses while operating them in real time—the functional equivalent of repairing one’s car while motoring down the expressway.

Today, the only NCMs in broad use are personal-selling approaches such as KAM; most others remain in pilot mode. But implementing even these incremental changes has proven challenging, especially in gaining the internal confidence to pursue a different set of tactics in satisfying the customer base.

“Adoption goes slowly with new tools because our own internal staff are not comfortable using the new tools,” one survey respondent told us. “This must be resolved if we are going to have any hope of getting our customers to adopt.”

On the content side, the most broadly applied variation has been the product budget/outcomes model. Nearly

85% of the executives we surveyed had at least piloted programs around this approach, and more than half had broadly applied the tactic as part of their overall strategies. Complementing that effort, about 70% had at least some experience with the innovative pricing/contracting model.

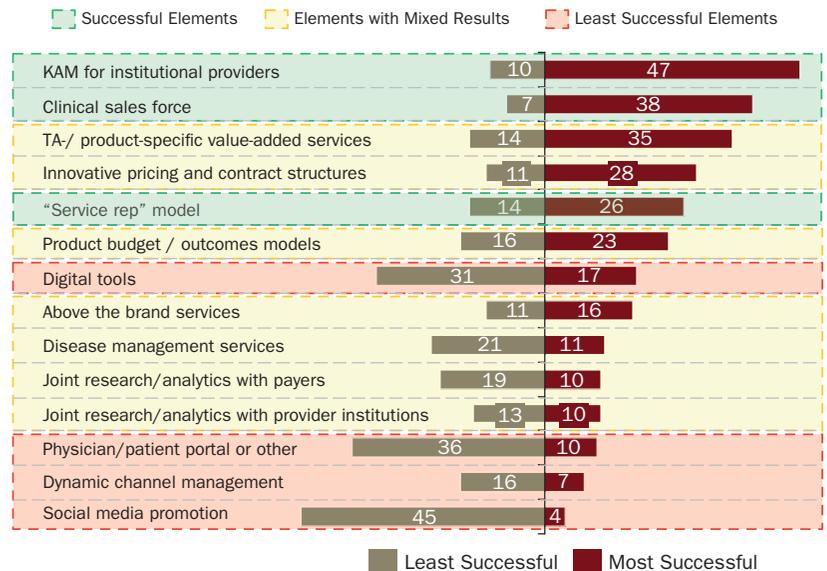
Generally speaking, however, broad adoption of new content approaches has lagged well behind those strategies focused on channels. Despite the fact that there has been fairly heavy piloting around the previously mentioned content strategies, the number of companies that applied these tactics only eclipsed a majority in the level of support in one instance—the product budget/outcomes model. Almost none of the other content-oriented commercial model levers have been applied in even a third of the cases, with collaborations falling at less than 25% for both payers and providers.

### Mixed results

While our survey highlights the wide assortment of strategies, in various forms

### Respondents selecting each element among the top 3 most or least successful NCM approaches applied by their organization

% of respondents (n=136 and 129 for most and least successful, respectively)



Source: PwC's 2014 Strategy & Global Pharma Marketing & Sales Study

and combinations, at play in the marketplace, it also clearly shows that, in most cases, the results have been decidedly mixed. The KAM strategies aimed at institutional providers and more clinical-specialist-led sales forces ranked as the most successful strategies employed. It is worth noting that these personal-selling variations, leverage the strengths of the traditional commercial model. The lingering question remains, however, as to how long the industry can continue its heavy reliance on these tactics, which, besides being more costly, present challenges in providing broad reach.

One of the survey's greatest surprises may be the executives' ranking of new technological tools, which they deemed among the least successful NCM elements. Pharma companies have devoted notable amounts of resources to digital tools, patient-physician portals and social media promotion, where many see "revolutionary" potential. Yet these areas have proved the least successful of any of the applied strategies, according to our respondents.

Though some of those surveyed appreciated the ability of digital tools

described as "highly appreciated by customers, but too expensive," and difficult to measure from an ROI standpoint. Both saw nearly equal levels of satisfaction and dissatisfaction. This contrast could indicate that success depends more on execution and is company- or brand-dependent. But, in any case, there is a fair amount of disagreement over what works.

Moreover, in the survey results and in our conversations with clients, we have found that quite often ROI is not consistently "measured." In those conversations, opinions have tended to be based less on actual data and verifiable ROI, and more on companies' impressions of how various commercial elements are performing. Given this dynamic, it is perhaps less surprising that the discussion of strategies and their efficacy breeds such disagreement.

---

*Pharma companies have devoted notable resources to digital tools, patient physician portals, and social media. Yet these areas have proved the least successful of any of the applied strategies*

---

This reliance comes as no surprise. While sales forces have shrunk and been outsourced, the industry remains dependent on direct sales forces. Almost all of our respondents have put in place some form of KAM strategy focused more on business-to-business interactions, where a team member represents the full portfolio and manages the relationship between large provider systems and the company.

The next iteration of this strategy will require companies to continue to bolster KAM approaches for institutional providers through better resource alignment, effective engagement from across the various pharma company functions, and development of more customer-centric offerings to gain access and influence.

But beyond the key account channel model and clinical sales rep approach, the results for the new offerings have varied. Respondents were far more comfortable identifying their successes based on channels of engagement rather than based on offerings.

to "Increase the speed and reach of communication dramatically," as well as the ability to better track customer interactions, others noted that the strategy remains "untargeted." Interviewees noted that so much of the digital effort never really "changed the market equation." They were simple additions or pushed the traditional personal-selling message online with little consideration for gaining customer demand for the information.

Further confounding the situation is the lack of standards by which to measure efficacy. As one respondent put it, the digital tools' "fanciness and superficial evaluation influence investment decisions," but that there remains a troubling lack of both focus in their application as well as "meaningful benchmarks to assess success."

Where the results were less clear for content approaches, however, was in the middle. Strategies such as above-the-brand services, and joint research/analytics, to name two, were

### **What now?**

The survey shows that executives will continue to invest in digital tools. The vast majority of survey respondents told us they were in the process of exploring alternative commercial elements, both broadly applied and piloted—a full 83% said they expect to further restructure their commercial model in the next two to three years. Clearly, they will continue to pilot new methods and spend more money as pharma executives continue to wrestle with NCM strategies—specifically digital strategies.

Though the industry has struggled to maximize the perceived potential of the various digital strategies it has attempted, and to measure their success, executives who responded to the survey indicated they would continue investing in digital tools. But even as they foresee proceeding further down this path, the executives continue to rate digital strategies as the least effective of those they have tried. Further, the respondents who had committed the most resources to those strategies reported the most uncer-

tainty as to their effectiveness. This is partly due to digital's relatively low cost coupled with its high potential. Hence, 65% expect to increase digital interactions, with support centered on digital tools. With further investment will also come a greater need for more accurate and agreed-upon ROI metrics.

Progress will likely remain incremental on this front, at least for now. But if there will be success, companies will need to do more than "add digital" as part of their strategy. They will need to look at how digital can really change the market dynamics for customers and how it will be in demand from these key stakeholders.

As always, the investment and divestment strategies companies employ will by necessity remain opportunistic, with eyes keenly trained on methods for achieving cost-effective growth. But in those areas where success remains elusive or, at the very least, debatable, companies will be required to continue in their efforts at redesigning offerings to better suit the market.

There are considerable resources already deployed around these strategies and with that comes a certain amount of inertia. Changing course will certainly not be easy. But that inertia creates the need to anticipate the changing landscape even sooner, lest companies be forced to turn on a dime somewhere down the road.

Following on that notion, and perhaps more importantly, leadership teams will be required to address the operational and institutional barriers that continue to block innovation and in many cases exacerbate the inertia.

There is a common misconception in the industry that the regulatory environment—with its increasingly difficult-to-navigate rules and the resulting upheaval in the stakeholder landscape—is primarily to blame for these internal blockages. The results of our survey show that, in fact, equal culpability lies

with the companies themselves, as they remain unsure of how to accurately determine the efficacy and ROI of the NCM strategies they attempt.

### Moving forward: Four recommendations

This year's survey results present a number of takeaways—but there is no one silver-bullet strategy to address them. Every potential solution that companies adopt moving forward must be tailored to suit the needs of their teams and, more importantly, the needs of their customers.

---

*Companies must never lose focus on the needs of customers—what will deliver the highest satisfaction, outcomes, and efficiency from their perspective*

---

If there is one clarion call—one first step we would recommend—it is for companies to fully embrace the alternative personal selling or KAM methods, if they have not already or if they've done so in a way that's not delivering on the expected value. It will be critical to continue evolving the capabilities here—including targeting tailored value propositions, fostering organizational alignment, and ensuring a better fit with local selling strategies.

Next, companies still struggling with the many moving pieces of the NCM should be judicious with resource allocation. Before spending millions of dollars on strategies such as new content/offerings, which may or may not prove effective in the long run, it's vital to understand which of those strategies have true innovative potential rather than continuing blindly down myriad paths. To that end, adopting and ensuring best prac-

tice execution in strategic moves that show the most promise, while also continually redesigning offerings and tools—especially digital tools which may not yet be delivering on their potential value—should remain key components of any new model.

Third, companies must never lose focus on the needs of customers—what will deliver the highest satisfaction, outcomes, and efficiency from *their* perspective. We have seen, in some cases, a tendency in large organizations toward navel-gazing. Even for those who have made tremendous strides with their account management, a better understanding of customers' needs can only aid future endeavours.

Finally, as we have noted, the market will continue to shift. Difficult as it may be, companies must continue to identify and address the operational and organizational barriers which stand in the way of more fully adapting to those shifts. Given the amount of resources necessary to bring new products to market, and allowing for the fact that effecting dramatic change within the pharmaceutical industry can often feel like steering an oil tanker, there is little need to further impede that process with internal barriers.

To sum up, taking full advantage of the potential of NCM strategies, pharma companies must evolve to a more sophisticated selling approach. Continued reliance on personal selling models may serve as a useful point of leverage in the near term, but a deliberate effort to improve customer value with a precisely targeted combination of NCM techniques will be necessary if pharma wants to achieve the revolutionary advancement that changing market conditions require. **PE**

---

**Rick Edmunds** is senior partner. He can be reached at rick.edmunds@strategyand.pwc.com. **Rolf Fricker** is partner. He can be reached at rolf.fricker@strategyand.pwc.com. **Stephan Danner** is partner. He can be reached at stephan.danner@strategyand.pwc.com. **Nelia Padilla** is principal. She can be reached nelia.padilla@strategyand.pwc.com. All with PricewaterhouseCoopers LLP

# Our Love Affair with Buzzwords

How pharma can finally put true meaning behind its juiced-up jargon and turn the bluster into reality

One unusual fact that I've learned from working in the life sciences sector for the greater part of this past decade is that we on the commercial side of the business have an unhealthy obsession with "buzzwords." From *Value Added "Beyond the Pill" Patient-Centric Services* to *Integrated Multichannel Customer Centric Closed Loop Marketing*, it often seems we spend more time marketing our own innovative ideas than actually innovating for patients, caregivers, and health-care professionals (HCPs). Even I admit that my own job title (see below) could be considered a case study in this "buzzword-driven" reality and, as a result, I'm often approached by individuals asking me exactly what it is that I do. Yet with all of these words, the reality is that, while we are making some progress toward patient and customer centricity, we are too often distracted by trying to "look" innovative. True progress tends to stall quickly. By focusing on some fundamentals, however, I have faith that we can not only turn these words into reality, but also utilize them to improve the overall perception of our industry.

## Case study

As a practical example, let's first look at the current industry buzzword of building "beyond

the pill" mobile health services through an admittedly generalized story (although one that I've both seen and heard numerous times from others in the industry):

*Product Manager X is tasked with developing an innovative "beyond the pill" mobile health service with a business case firmly rooted in improving patient compliance*

---

*The truth is, getting serious about developing these "value-added services" is hard work...almost as hard as launching a new treatment*

---

*to treatment. Said product manager calls up digital agency Y that develops the Mercedes of treatment trackers designed using the most cutting-edge wisdom in behavioral psychology, user experience, and graphic design. Product Manager X and his director decide to limit the application's usage to only patients that are currently being prescribed their product in order to meet the numbers promised in the business case. The application is launched on the App Store and promoted by the field force with business cards that include the top-secret login code for patient access. In the end, the app is used by only a handful of patients, yet Product Manager X is lauded for this in-*

*novative work, and the agency walks away with a few industry awards and case-study slides to use for future pitches.*

So how could the above have been turned around? First, the reality that should have been understood from the beginning was that launching this service was, in itself, launching a new product. There were competitors, market dynamics, product positioning, and a limited number of unmet patient needs. A quick search on the App Store (Google or Apple) would have delivered double-digit results of pre-existing patient service apps in the therapy area, many

of which were actually developed by patients for patients. Equally, if the business case for the service was developed solely to maintain treatment adherence (thereby increasing prescriptions), the application was already doomed to fail as this goal was never a perceived unmet need by the end-user; so much for "patient centricity."

The truth is, getting serious about developing these "value-added services" is hard work... almost as hard as launching a new treatment. It's no wonder that the likes of Google, Apple, and the big Silicon Valley venture capital firms are betting heavily on the future of digital health because there is so much opportunity to be had. The advancements in sensors to detect an increasing number of symp-



**Timothy White** is Senior Director & Head of Global Customer Interaction Management, Lundbeck. He can be reached at TIMW@lundbeck.com.

toms or other health data points is truly remarkable and when you combine this technology with the existing mobile devices that we all carry with us, the future for digital health technology is very bright.

That's why we need to start thinking about these services as the products are being developed in the labs and not as a marketing add-on at the end of the value chain. Moreover, we need to build the proper skills, processes, capabilities, and reward systems in our organizations to do this and, only then, will a buzzword like "beyond the pill" truly take on a value-adding role for patients as the words so confidently suggest.

### Lost in translation

The other area where I see these buzzwords prevailing is in the current innovation in how we communicate our products. Terms like "closed loop marketing" and "multichannel" are prevalent in almost any chief marketing officer's handbook these days, but if you ask many of them, they'd be hard-pressed to quickly explain the value or how it drives customer centricity. If you look at the situation from the outside, there actually is a lot of value to be derived from all of this new technology provided by innovation-driven cloud technology companies. Our sales teams can now have the most up-to-date information available in real time on mobile devices. Marketing content and other resources can be tailored to customer needs, and we now have many new channels of communications to improve communications to our customers.

But if companies just use all this powerful technology to bombard our customers with

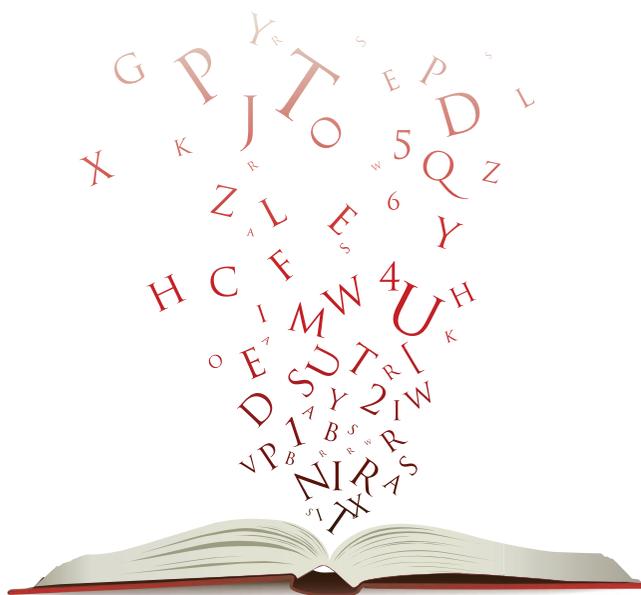
the same brand messaging or to monitor and measure our sales teams to the point where every single slide shown is being looked at for potential commercial impact, then we're simply missing the point. HCPs are being asked to see more and more patients with less time, they're being bombarded with information, so it's no wonder that it's becoming harder for our field teams to gain access to physicians—closed loop marketing and iPads alone won't fix that.

Now, if this technology is harnessed to create a more valuable customer experience where sales teams provide relevant, timely, and iterative information to HCPs through their channels of choice, then the concept of "customer centricity" starts to shine. I have seen this work, and it is indeed inspiring when a physician walks away from an interaction with a pharmaceutical company like it was his or her preferred bank, retailer, or other customer-experience leader. The technology is here now. It's now more about how we leverage all that it offers. From marketing to sales to analytics to general

management, building a great customer experience starts with people and is only empowered by the technology. We need to focus on building new internal capabilities and shifting the overall mindset on how we develop insight, plan, and execute our commercial strategies to truly become customer centric.

### Beyond the buzz

Let's imagine a world where all these common buzzwords were real, where we were developing truly innovative services in combination with our novel new compounds that were improving or extending the lives of people; where pharmaceutical representatives were trusted advisors to medical professionals, relied upon to both educate about new treatments AND facilitate and coordinate various services for patients. While I don't think we'll ever see patients or HCPs lining up for the release of Phase III data the way that fan-boys do for the release of a sexy new Apple gadget, I have to imagine that if these visions became a reality, the truth wouldn't be so far off. 



# Have Your Eggs, and Your Statin (and Your PCSK9 inhibitor, too?)

In the popular media, dietary guidelines make clickbait when “LDL” is in the title. Last month, the HHS 2015 Dietary Guidelines Advisory Committee revised America’s preferred diet, saying that the amount of cholesterol consumed is not as big of a factor as previously believed. Chicken coops nationwide reportedly plan to up production immediately.

Cholesterol is back. Clearly, it didn’t go anywhere as both a massive public health issue for millions of American or as an enormous market with billions in revenue for statins and other dyslipidemia fixers. But as a headline grabbing, controversy stirring money pot with momentous scientific innovations and big Pharmas and big payers strategizing like chess grandmasters or pro poker player—for drugmakers and their financial backers and detractors, cholesterol is back, with a vengeance.

Laws of big numbers most certainly apply. Brands have competed and gone generic; sheer numbers are staggering. Close to a third of Americans are at elevated LDL levels and less than half are seeking treatment, says the CDC. The market is massive, second only to hypertension, with 24.4 million on-therapy patients treated for cholesterol in 2013, according to a 2014 IMS report.

The impressive numbers do signify progress, of a sort. The CDC notes that 96 million visits to doctors’ offices in 2009—9.2% of all visits—included cholesterol tests. A significant portion of the populace is aware, treating, and improving their cholesterol measures.

The numbers remind us that cholesterol is a health issue that the biopharma industry can impact positively. However, as the new dietary guidelines explain, cholesterol is a multifactorial disease with genetic involvement and environmental determinants. Western citizens with sedentary lifestyles and diets high in sugars and saturated fats, regardless of the new thinking on cholesterol counting, are largely guilty.

Hence, criticisms echo from some physicians. A panel at last fall’s 2014 Prix Galien Forum debated the industry’s role, with some saying it has a role in manipulating treatment guidelines, medicalizing an issue that should be more about healthy living.

In addition, some experts, like blogging doctor, cardiac electrophysiologist Dr. John Mandrola, write of concerns over irrational exuberance over statins and his growing concerns of using them to preemptively treat heart disease, especially considering side effects, he says, which are clearly issues in the real-world treatment setting.

“No statin drug has ever been compared to lifestyle interventions for the prevention of cardiovascular disease,” Mandrola writes.

With that debate far from settled, prepare yourself for a significantly more complicated marketplace. 2015 promises two big new players in the form of PCSK9 (proprotein convertase subtilisin/kexin 9) enzyme inhibitors. Sanofi and Regeneron’s *Praluent* (alirocumab) will get a jump-start on Amgen’s evolocumab, thanks to its ace in the hole, a priority review voucher bought from Biomarin for \$67.5 million.

Payers are holding their collective breath for the biologics, which could come to the market at a price tag floating in the range of \$10,000 a year, for huge populations that would be chronically treated.

Actually, holding their breath is far from an accurate description of 2015 so far. Licking their wounds from the year of *Sovaldi*, payer executives at Express Scripts and CVS have fired vocal shots across the bows of big Pharma and the investment community expecting big profits.

In January, Express Script’s CEO George Paz took a dig out of the PCSK9 bull market when he promised that payers would take action and force the fight over pricing the cholesterol-lowering biologics. Some lessons have been learned from the hepatitis C market and will be applied, no doubt.

Then, mid-February, four CVS Health executives sent their Valentine’s in the form of a post on *Health Affairs* Blog, in which they question the ability of the healthcare system to absorb the sticker shock that might be approaching. Though their estimates may be hyperbolic (Forbes’ Larry Husten called the CVS executives’ guess of \$150 billion annual impact “a bit silly”), the quantities will undoubtedly be massive.

The huge numbers do, however, cause us to ponder questions that might be too obvious. In an industry that is moving toward a patient-centric mindset, what efforts could be made by the industry to minimize the deluge? Does being patient-centered mean approaching the patient’s lifestyle more aggressively? And if tax payers are footing the bill, at what point does paying for your fellow citizen’s lifestyle become too much? Or have we already been down this road? Examples include smoking, alcohol, etc.

Even the great innovation that PCSK9 inhibitors promise to be, it still feels a bit like a Band-Aid or a finger plugging an imploding dam. **PE**



Casey McDonald is Pharm Exec’s Senior Editor. He can be reached at [cmcdonald@advanstar.com](mailto:cmcdonald@advanstar.com).

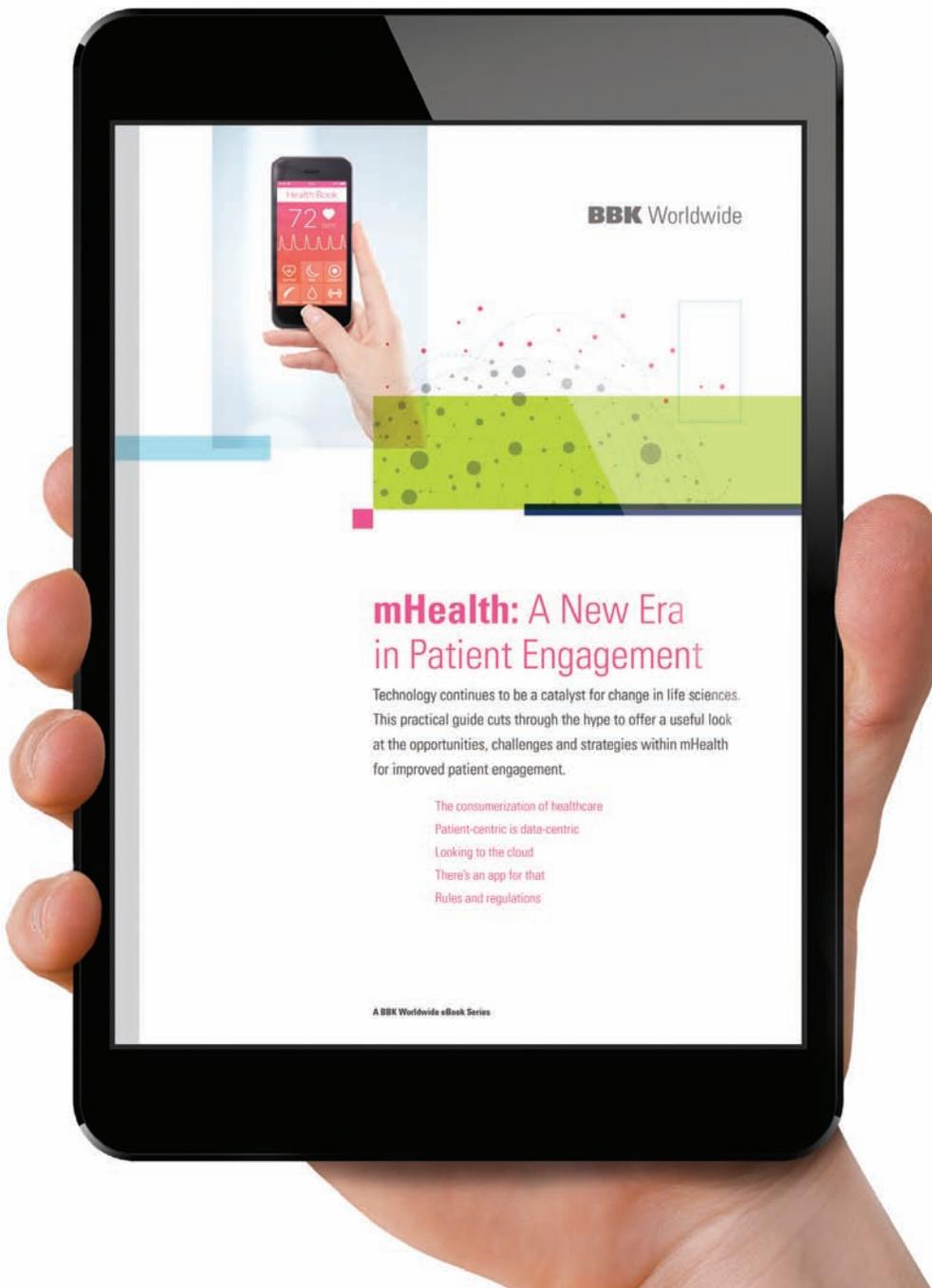
# mHealth +

## Patient Engagement

mHealth continues to drive seismic shifts in the way we collect, view and share data – data that is key to improved patient care and reduced study timelines. Download BBK Worldwide's essential guide to this new connected health model, and learn how the right mHealth strategy can impact your patient recruitment and engagement programs.

**DOWNLOAD YOUR FREE COPY TODAY!**

**[bbkworldwide.com/mHealth](http://bbkworldwide.com/mHealth)**



# DOWNSTREAM

To get new biotech or novel therapies to market takes starting at the beginning. It takes a partner with the experience, resources and expertise to advance promising ideas into fully realized solutions. It takes a global healthcare solutions leader dedicated to enhancing patient care through end-to-end solutions for manufacturers, pharmacies and providers. It takes persevering upstream and executing downstream. It takes AmerisourceBergen.



[ItTakesAmerisourceBergen.com](http://ItTakesAmerisourceBergen.com)