BY DENISE MYSHKO

» RESEARCH

ADDRESSING
THE INNOVATION CHALLENGE

There has been a great deal of discussion related to the challenges hampering **INNOVATION TO CREATE DIFFERENTIATED MEDICINES**.

Never before have research and cutting-edge science been more important to ensure a steady flow of innovation.

Much has been made of the industry's decline in R&D productivity over the last few years. While the number of new drug applications (NDAs) under review at the Food and Drug Administration declined 47% from 1995 through 2007, according to data from Parexel Consulting, there was a 22% surge in original NDA submissions during 2008.

The FDA entered 2009 with 147 pending NDAs under review, up sharply from 86 a year earlier and the highest number since 1995, according to Parexel Consulting.

Industry experts interviewed by PharmaVOICE believe that while obstacles remain for bringing innovative products to market, the industry is adapting and working to address those obstacles.

What is needed is a mind shift with regard to how to move innovation to the next level.

"The good news is that innovation is here," says Alice Jacobs, M.D., CEO of Intelligent-MDx. "The technologies necessary to bulk up drug pipelines have already been discovered. The innovation challenge is not technical, but philosophical. Without the co-discovery, co-development, and co-marketing of companion diagnostics and therapeutics, we will fail to maximize benefits to all stakeholders."

Laurie Halloran, president and CEO of Halloran Consulting Group, contends that the pipeline is not dry; rather, there is a huge disconnect between the discovery engines at small companies and academia and the late-stage development machine in big pharmaceutical companies.

"Innovative medicines are being discovered now in top universities; they just get stuck in the valley of death between the bench and patient," she says. "Discoveries are not the challenge; shepherding the best of them to proof-of-concept in development is. Companies must get more sophisticated through

Kenneth Aldrich
International Stem Cell

"One of the most rapid accelerators of new product pipelines would be for larger pharma companies to increase their funded and collaborative research with early-stage companies."

Simon Higginbotham
Kendle

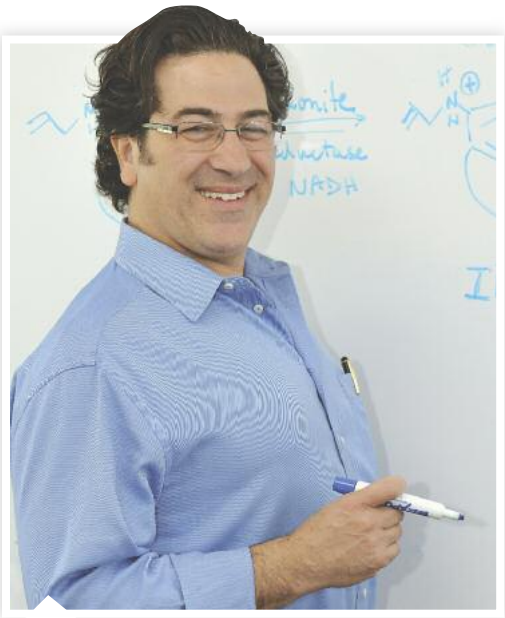
"The need for pipeline productivity will lead to increased rationalization of portfolios and greater up-front planning. Earlier proof-of-concept studies for more accurate go/no-go decisions are a must."

Jesse Bowden
Biomedical Systems

"The next generation of great discoveries for innovative medicines will come from nanotechnology, personalized medicine, gene-therapy research, long-distance robotic surgery, and RNAi/siRNA drug development."

Glen de Vries
Medidata

"The current crop of emerging techniques include standards, integrations, visualization, and algorithmic mining; these will need to be perfected and built up to address the tasks ahead."



Dr. Julian Adams
Infinity Pharmaceuticals

"Companies need to continue to embrace the approach of 'fearless chemistry,' while also devoting time, energy, and mindshare to integrating their internal teams."

innovative business models that support lean development."

PARTNERING FOR CHANGE

Industry leaders acknowledge the critical role played by R&D partnership.

Joseph Pieroni, president and CEO of Daiichi Sankyo Inc., says companies will be required to supplement their own R&D efforts through external alliances and in-licensing of promising compounds.

"There is definitely a convergence of technologies and advances in each area that will lead to new developments in the healthcare industry," he says. "Collaboration with other companies that already have these technologies is the best way to bring outside expertise into an organization and drive innovation."

Mr. Pieroni points out that there are more products in development now than in the past.

"The areas in which many companies are choosing to innovate and develop new drugs have shifted, for example, from cardiovascular drugs to oncology medicines," he says. "The real challenge is to find true and definitive incremental benefits over products that currently exist. The current dynamics of the industry and the economy will continue to encourage companies to embrace strategic partnerships as an effective way to gain access to new technologies, add needed resources, and expand the number and diversity of R&D initiatives, as well as better balance the inherent risks in drug development."

The partnered approach becomes more important given the need to focus on riskier R&D, especially as low-risk projects are increasingly shown to yield drugs with little market advantage, says Robert Dickinson, head of the life-sciences practice at Grail Research.

> Marketing exclusivity periods for first-in-class drugs have fallen dramatically in recent decades, from a median of 10.2 years in the 1970s to 2.5 years in the early part of this decade.
>
> TUFTS CENTER FOR THE STUDY OF DRUG DEVELOPMENT

"We see large pharmaceutical companies taking more of an 'investment portfolio' approach to their pipelines by working with a wide range of R&D partners to have access to a larger pool of opportunities and to better manage risk," he says. "R&D, while still a core function, will experience a reduced head count as the industry continues to consolidate."

Discovery efforts, particularly those in big pharma, will increasingly focus less on primary scientific research and more on the analysis and assessment of licensing and acquisition opportunities, Mr. Dickinson says.

"This move will be accelerated by the larger industry's move to increasingly emphasize core competencies, such as clinical trials management, marketing, and access while tapping more outside sources for R&D innovation," he says. "Consistent with this trend we will also see more active partnerships between R&D, business development, the biotechnology sector, and members of the academic community."

Ms. Halloran agrees that big companies will increasingly partner to advance development.

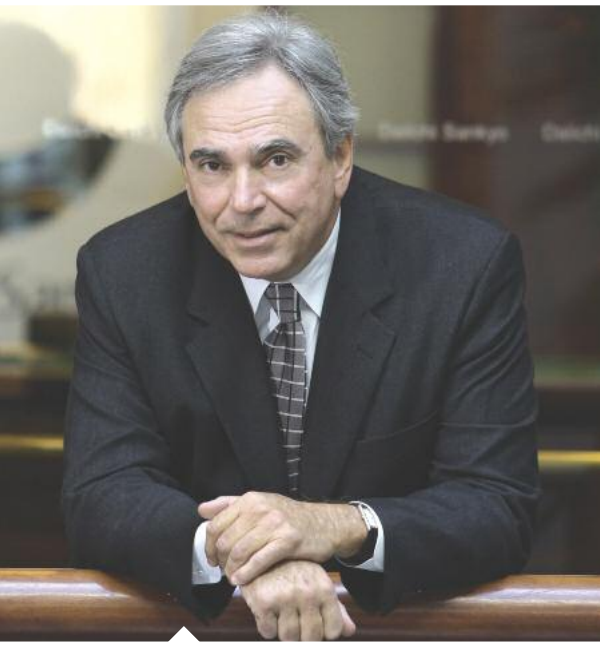
"Expansion of nimble early development spinoffs from global companies will be augmented by the virtual development model to expand the number of later-stage candidates," she says. "Early-stage development will not be the focus of big companies. Nimble virtual teams of senior-level experts will work on multiple platforms using flexible outsourcing partners. Product development companies will be established and funded through new channels to develop high potential candidates to a predefined proof of concept and divestiture to a big biopharma partner with shared risk and reward."

Alistair Macdonald, executive VP, global services, at INC Research, believes there will be more of a separation between "R" companies and "D" companies.

"I believe pharmas will be more willing to

Robert Dickinson
Grail Research

"The most pressing factors impacting large pharma R&D include: less real innovation due to the industry's continued natural inclination to invest in low-risk projects; greater need to evaluate licensing/acquisition targets in the small and midsized biotech sector; and a steady decrease in R&D head count due to decreasing performance."



Joseph Pieroni
Daiichi Sankyo

"The current dynamics of the industry and the economy will continue to encourage companies to embrace strategic partnerships as an effective way to gain access to new technologies, add needed resources, and expand the number and diversity of R&D initiatives."

Dr. Michelle Dipp
Centre of Excellence for External Drug Discovery, GSK

"Mutually beneficial collaborations between academic labs, small biotechs, and major pharmaceutical companies will grow in importance."

PERCENT OF SUBMITTED NDAS AND NMES OBTAINING PRIORITY STATUS, FY2003-FY2009*

	NDAs	NMEs
FY 2003	19%	43%
FY 2004	22%	53%
FY 2005	30%	50%
FY 2006	23%	33%
FY 2007	20%	31%
FY 2008	22%	—
FY 2009*	8%	—

Note: * through March 31, 2009
Source: Parexel Consulting

buy in new compounds from academia," Mr. Macdonald says. "We are already seeing mid-level pharma companies taking this approach. They don't conduct their own research; they just focus on the development."

Increasingly, companies should consider teaming up for products in earlier stages of development, says Kenneth Aldrich, CEO, International Stem Cell.

"One of the most rapid accelerators of new product pipelines would be for larger pharma companies to increase their funded and collaborative research with early-stage companies," he says. "That goes counter to current trends, which are to wait until a company is in middle- to late-stage trials, then purchase the company or technology. If large companies adopt more of a venture-capital-like strategy, some money will be wasted on failures, but the cost of those will be relatively small and more science will enter the pipeline at a far lower cost than by continuing the present model. Since the failure rate, even in Phase II or III trials, is still high, why not reduce the cost of picking the best candidates by participating at an earlier stage in sorting and funding startup technologies."

Already, collaboration has become a key component for large pharma companies and is predicted to grow.

"The next generation of new treatments will come from multiple sources — from academic labs, small biotechs, and major pharmaceutical companies," says Michelle Dipp, M.D., Ph.D., VP and head of U.S., GlaxoSmithKline's Centre of Excellence for External Drug Discovery (CEEDD). "Mutually beneficial collaborations between these entities will grow in importance, and at the end of the day it will be these scientific advances that will help improve the lives of patients. Companies can look to new collaborations as a means to bolster their pipelines. At GSK, the CEEDD is looking to link up with biotech companies to bring important new medicines into the organization, and we're constantly in search of and evaluating innovative technology."

The economic climate has been a key driver for partnerships and collaborations and will continue to feed the industry, along with M&A activity, according to Ryo Kubota, M.D., Ph.D., chairman, president, and CEO of Acucela.

"Smart larger companies will partner with dynamic biotech collaborators with exciting pipelines to advance their R&D and to fuel the development of new, breakthrough drugs," he says. "Strategic partnerships are a win-win for both parties. Often, the pharmaceutical company obtains access to innovative science while biotech companies gain access to funding that is essential to advancing their pipelines."

Michael Harte, founder and president of the Harte Group, believes that many incubators and smaller companies hold some of the novel medicines needed to move the pipelines forward.

"The challenge in the industry today is the continued escalating costs that come from the development of these compounds," he says. "The industry needs to hit singles and doubles rather than always swinging for the fences. Singles and doubles typically produce more runs, more frequently."

As has often been said by biotech leaders, it is difficult for large pharma companies to innovate.

"Biotech organizations are proven pioneers and unafraid to pursue untapped areas where there is significant unmet medical need," says Richard Pops, chairman, president, and CEO of Alkermes. "When we look back five years from now, biotech companies will be pursuing blockbuster drugs, which were historically

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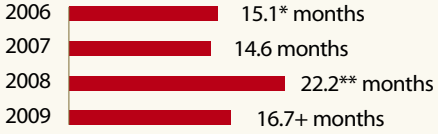
Dr. Alice Jacobs
IntelligentMDx

"Safety and efficacy are most precisely maintained by using clinically impactful molecular diagnostics to monitor how a drug is affecting the patient over time. The capabilities to produce these outcomes exist."

THE FIRST-CYCLE REVIEW IMPERATIVE, 2006-2009

Approval Time Difference Between NMEs Receiving First-Cycle Approvals and Those Requiring Multiple Cycles (Two or More)



Notes:

* Excluding one extreme outlier, 6 year review of Pylera

** Excluding multi-cycle 60.3 month review of Vasovist

+ For NMEs approved through June 30, 2009

Source: Parexel Consulting

Dr. Richard Eglen
PerkinElmer

"A best practice for improving innovation will be increased outsourcing of previously core in-house R&D activities, such as HTS, lead profiling, medicinal chemistry, ADME/tox optimization, and biomarker detection."

considered the domain of big pharma companies. Biotechs with the financial resources and scientific expertise will be able to innovate in some of the major chronic diseases faced by millions of people every day, such as diabetes, obesity, addiction, and depression."

According to Mr. Macdonald, one of the biggest impediments to innovation is the industry itself.

"The drug development industry has traditionally been slow to adopt any type of change, and likewise is very risk averse," he says. "The mindset needs to be more progressive and open to new approaches and technologies. The driver for this type of change will come from many factors, including pricing pressure."

Mr. Harte agrees, adding that two of the greater challenges inherent in fostering innovation are the lack of flexibility within the research process and the misaligning of incentives toward quantity versus quality.

"Study teams need access to the newest technologies and services that provide them with the greatest opportunity for success," he says. "Many teams are locked into preferred agreements with companies that cannot offer the flexibility and creativity needed to execute their projects."

NEW DISCOVERIES LEADING R&D

Industry experts PharmaVOICE interviewed say new technologies, biotechnology, personalized medicines, RNA expression, and stem cells are a few of the areas that will generate innovation.

"Adoption of new technologies that can add value to drug discovery, both in the instrumentation area and in the biology/chemistry area, will help foster innovation," says Richard Eglen, Ph.D., president of bio-discovery at PerkinElmer. "New technologies need to show incremental increases to efficiency of drug discovery, rather than be changes 'on the margin.' This requires rigorous evaluation of the new technology and rapid assessment of its utility. It also requires close collaboration of the drug discovery organization with the technology development companies to develop innovation in the areas that are needed."

Areas Dr. Eglen believes will gain further traction include cellular imaging, label-free detection systems, cell-based assays, fragment-based chemistry, in silico screening, in vivo imaging, microfluidics, 3D-tissue assemblies, and stem cells for high-throughput screening.

"In the stem-cell arena, there were big breakthroughs this year with non-DNA-based methods, specifically using proteins and small molecules, to derive induced pluripotent stem cells (iPSCs)," says Paul Grayson, president and CEO of Fate Therapeutics. "I expect great strides will be made toward industrializing this technology to produce a pharmaceutical-grade iPSC. Being able to reprogram a cell without genetic modification at commercial-scale quantity, consistency, and quality will have a large impact on the advancement of this promising technology for drug discovery and cell therapy."

Another hot area of technological advances relates to biomarkers, and Dr. Eglen says there are two aspects to this issue.

"The first is coming to an agreement on the key biomarkers required to both predict compound efficacy as well as to predict compound toxicity," he says. "The second is related to the actual technology requirements to detect such biomarkers. It appears that there is movement on the first area with several companies defining the biomarkers needed for their own proprietary clinical studies, as well as the fact that regulatory agencies are moving to define those biomarkers required for use in clinical studies.

"In the second area, technology development is moving rapidly, both in terms of high throughout assays to detect key biomarker proteins present in biological fluids such as blood or serum, as well as nucleic acid-based assays to detect key changes in mRNA or DNA," Dr. Eglen adds. "Converging with these trends is the development of technologies around cell isolation and microfluidics that will enable precise detection in small and rare clinical samples."

Biomarkers come to the fore in personalized medicine, a concept that Mr. Pieroni says is now firmly entrenched in the pharmaceutical industry.

"As scientific advances improve our understanding of how drugs work differently in different patient groups, biomarkers that predict safety and efficacy response will allow us to better target therapies for individuals, thereby

integrated intelligence

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PAREXEL CONSULTING

Richard Pops
Alkermes

"The biotech industry will be viewed by the public as a wellspring of new ideas and treatments for the most important diseases and medical challenges we face."

increasing a drug's effectiveness while lowering the risk of unwanted side effects," he says. "The hope is that with targeted therapies we can use the convergence of new technologies to increase the safety and efficacy of products and improve the benefit-risk profile for patients."

Harnessing the therapeutic power of biomarkers to drive personalized medicine will require the industry to make some process changes, says Julian Adams, Ph.D., president of research and development and chief scientific officer at Infinity Pharmaceuticals.

"There's great promise there, but there are also so many challenges in terms of the application of cutting-edge research and breakthrough science to real-world clinical development," he says. "If we can focus our energies on pulling these innovations into clinical trial design and R&D process, we may be able to push through and truly change the way drugs are developed."

Dr. Sanjay Parikh
Indegene

"A better understanding of the genetic etiology of diseases will result in disease-modifying therapies."

Mr. Pieroni says if companies began identifying promising technologies now, the industry wouldn't have to wait long for personalized medicine.

"Genomics also help us understand how individuals process medication," he says. "Genomics will be important in identifying patients who will not respond to or are likely to have side effects from medication. Again, this could lead to better efficacy and safety profiles. The convergence of technologies such as genomics, embedded devices, artificial intelligence, virtual reality, nanobiology, and supercomputers will create a new healthcare landscape."

John MacPhee, president of Strativa Pharmaceuticals, says technological advances such as genetic mapping are already changing the way diseases are diagnosed, treated, and managed.

"The one-size-fits-all approach to medicine is giving way to a more individualized approach to improve outcomes, compliance, and reduce costs," he says. "There will be fewer blockbuster drugs in the future. These new, evolving techniques are allowing researchers to better understand the underlying causes of diseases — for example, genetic disposition — so that targeted therapies can be developed to help patients better manage their condition, thereby improving outcomes and quality of life."

This is also true with drug delivery technology, which is becoming more and more sophisticated, Mr. MacPhee says.

"Advances in drug delivery provide patients with new, more efficient, and convenient delivery options for proven molecules that can improve efficacy, safety, and compliance," he says.

Sanjay Parikh, Ph.D., director of Indegene, agrees that a better understanding of genetic etiology of diseases will result in innovative disease-modifying therapies.

"In the short term, companies can improve innovation through systems biology and pathway-based research to explore additional indications for existing therapies and identify logical and interesting drug combinations to

Michael Harte
The Harte Group

"Full-service, build-it-here models will disappear as the expanded use of niche or functional services begins to escalate. These vehicles provide for greater flexibility, more creativity, and more transparency in the execution of various service/technology uses."

Alistair Macdonald
INC Research

"The biggest impediment to innovation is the industry itself."

improve compliance rates and cost," he says. "In the long term, companies need to invest more aggressively in pre-proof-of-concept innovation. Companies will also have to redesign their R&D programs to yield go/no-go decisions much faster — even in silico — wherever possible; focus on first-in-class products rather than best-in-class products; and put greater focus on health economics and outcomes research-driven decisions."

Dr. Jacobs says pharma will need to accept that personalized medicine supports safer and more effective health outcomes, and, as a result, treatments may become more targeted.

"This means embracing diagnostics as part of the discovery process, to streamline enrollment in clinical trials, and to incorporate the utilization of companion diagnostics into the drug label claims," she says. "Safety and efficacy are most precisely maintained by using clinically impactful molecular diagnostics to monitor how a drug is affecting the patient over time."

BEST PRACTICES FOR SUCCESS

Aligning processes is as important as product innovation. Dr. Adams says companies need to continue to embrace the approach of "fearless chemistry," while also devoting time, energy, and mindshare to integrating their internal teams.

"I'm a scientist, but to do my job effectively so that we can discover the next compound that could move the needle, I need to understand how our overall team — and industry — is working to partner novel programs, collaborate, and design the most efficient R&D organizations possible," he says. "The same is true for our business team. They need to understand and appreciate what the R&D team is doing and why. Only then can we continue to build a sustainable pipeline and grow the business."

Simon Higginbotham, senior VP and chief marketing officer at Kendle, says the need for pipeline productivity will lead to increased rationalization of portfolios and greater upfront planning.

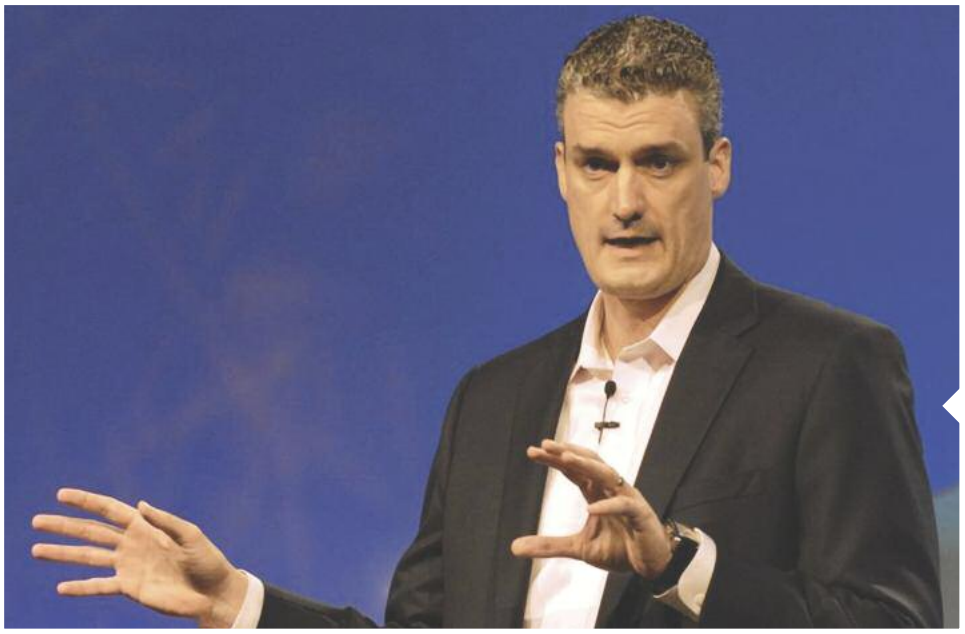
"Earlier proof-of-concept studies for more accurate go/no-go decisions are a must," he says. "Customers will be increasingly focused on their core competencies and will turn to CROs as strategic partners to drive efficiencies and add value to the clinical development process."

Jesse Bowden, president of imaging services at Biomedical Systems, agrees that more outsourcing to CROs and taking trials globally will improve innovation.

"Multinational companies will have to target more resources to improve pipelines," he says. "Also, the use of electronic healthcare data and payer data to identify the right patients and investigators for clinical trials will help finish trials on time and bring drugs to market faster."

Glen de Vries, president of Medidata Solutions Worldwide, says biopharmaceutical companies are overwhelmed by cascades of data, from discovery models, genomics sequencing, and provider and insurer data, as well as clinical trial results.

"To a large extent, the future of many companies will depend on their ability to derive meaningful patterns from this information as



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Dr. Ryo Kubota
Acucela

"Collaborations in today's biotech and pharma world are essential, and innovation is truly a global endeavor. The most successful partnerships will be those focused not on geography, but on creating new treatment options that will result in improved care for patients."

John MacPhee
Strativa Pharmaceuticals

"The new product-sourcing model will continue to be critically important. Whether sourcing products through internal R&D or through business development, the appropriate selection of new product targets for development is crucial for the long-term success of pharmaceutical companies."

well as develop commercial applications and/or operational efficiencies," he says. "The current crop of emerging techniques includes standards, integrations, visualization, and algorithmic mining and will need to be perfected and built up to the tasks ahead."

Tom Russell, general manager of enterprise solutions at SciQuest, says most global pharmaceutical leaders have already moved to achieve what will increasingly be seen as the hallmark of a successful global R&D process.

"They are making a concerted effort to apply strategic sourcing techniques honed in the manufacturing operation to the indirect supply chain that serves R&D," he says. "These efforts not only significantly lower the cost of R&D, but also remove what for many research operations represents the most time-consuming and problematic administrative task: the ad hoc purchase of new supplies as experiments evolve."

From a technology standpoint, Mr. Higginbotham says advances in EDC and data analysis technologies have increased the speed and accuracy of data collection, making adaptive design more practical.

"This in turn accelerates decision-making and the overall development timelines, reduces patient exposure to experimental drugs, and increases safety," he says. ✦

PharmaVOICE welcomes comments about this article. E-mail us at feedback@pharmavoice.com.

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EXPERTS ON THIS TOPIC

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RICHARD F. POPS. Chairman, President, and CEO, Alkermes Inc., a fully integrated biotechnology company committed to developing innovative medicines. For more information, visit alkermes.com.

TOM RUSSELL. General Manager of Enterprise Solutions, SciQuest Inc., which provides procurement automation and supplier enablement solutions. For more information, visit sciquest.com.

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SPEAKERS

Mark Sales
Head of Global Stakeholder Management
KantarHealth

Amy Krane
Director, Healthcare Solutions
TNS Cymfony

Gary Bartolacci
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BY DENISE MYSHKO

» RESEARCH

INNOVATION: AREAS FOR GROWTH

BIOTECHNOLOGY, PERSONALIZED MEDICINES, RNA EXPRESSION, AND STEM CELLS are a few of the scientific areas that will lead to innovative new products.

Dr. Tim Bertram
Tengion

"Regenerative medicine could truly revolutionize how we treat organ failure throughout the body by creating new organs and tissues."

Dr. Michael Chang
Optimer Pharmaceuticals

"With pharma hungry to fill pipelines and biotech pushing new compounds into late-stage development, anti-infectives are one of the hottest market segments to watch."

Stephen Simes
BioSante Pharmaceuticals

"Unfortunately, it seems that future development will be limited by the rather myopic view among some that only oncology, diabetes, obesity, and cardiovascular medicine are the projects worth funding."

PHARMA SUCCESS RATES

- Nearly three-quarters of the drugs in the portfolios of the top pharmaceutical firms that reached clinical testing from 1993-2007 originated in and were developed by the firms.
- Of six specific broad therapeutic categories analyzed, oncologic/immunologic and central nervous system (CNS) had the greatest number of drug candidates entering clinical testing over the 1993-2007 period.
- For the top 50 firms, the annual rate at which drugs enter clinical testing increased 31% from between 1999 and 2001 to between 2002 and 2007.
- Systemic anti-infectives had the highest clinical approval success rate for self-originated new drugs, with more than one-quarter of the drugs entering clinical testing during 1993 to 2004 estimated to receive U.S. marketing approval.
- Less than 10% of cardiovascular and CNS drugs entering the clinical testing pipeline during 1993 to 2004 are estimated to attain U.S. marketing approval.

Source: Tufts Center for the Study of Drug Development

Innovation in R&D has the potential to bring big changes to many areas, industry experts observe. Sylvia Miriyam Findlay, programme leader, pharmaceutical and biotechnology, healthcare, Europe, at Frost & Sullivan, says microfluidics and nanochemistry are set to bring in great advances in the field of drug discovery.

"RNA and its role in gene expression will also provide greater understanding of diseases," she says. "These will be areas of interest among the research community. Oncology and metabolic disorders will have prime focus in research, with biological drugs driving growth in the pharma industry."

Richard Pops, chairman, president, and CEO of Alkermes, says in addition to the quest for novel drugs from new classes of medicines, such as RNAi and epigenetics, there are compelling ways to develop new molecules that are built on existing science and chemical matter.

"It is possible to develop an innovative medicine that has important clinical value, without starting from 'square one' in the drug discovery process," he says.

Steve Worland, president and CEO of Anadys Pharmaceuticals, says new technologies alone will have little impact as they are gradually incorporated with only modest incremental effect on R&D efficiency.

"The days of thinking new technology itself fundamentally changes the capital efficiency of R&D in a positive way are thankfully over," he says. "A distributed model of situation analysis is a good practice, wherein all the best ideas from all the best people really impact final decisions about where and how to invest."

Kenneth Aldrich, CEO of International Stem Cell, says stem-cell research and nanotechnology, both individually and in combination, are likely to change the treatment of chronic diseases and the field of diagnostics.

"One area of almost certain rapid growth will be stem-cell-based treatments for retinal diseases, liver disease, heart disease, and some of the central nervous system diseases," he says. "We already know that human cells can be transplanted effectively to treat these diseases, so half the battle is already won. The issues remaining are mainly immune rejection and an adequate supply of cells."

He says as stem cells are developed into specific cell types to replace cadaver donations, the market will expand rapidly.

"There will still be issues with tissue rejection, but methods of cell creation that either create a 'bank' of available cells to match immune systems of large population groups, for instance parthenogenetic stem cells, or individualized

TRENDS IN MARKET EXCLUSIVITY

- Marketing exclusivity periods for first-in-class drugs have fallen dramatically in recent decades, from a median of 10.2 years in the 1970s to 2.5 years in the 2000-to-2003 period.
- Average time between first and second follow-on drugs fell even more rapidly — from a median of 16.1 years in the 1960s to 1.1 years between 2000 and 2003.
- Nearly one-third of all follow-on drugs have received a priority rating from the FDA.
- Since the early 1990s, 90% of follow-on drugs had initial pharmacologic testing and 87% were in clinical studies somewhere in the world before the first-in-class drug approval.
- Patent filings for follow-on drugs often occur in advance of first-in-class patent filing.

Source: Tufts Center for the Study of Drug Development

Dr. Jason Hwang
Innosight Institute

"Longer term, widespread diseases with poorly understood mechanisms will undergo more precise definition and treatment, transforming diseases and our ability to cure them."

Sylvia Miriyam Findlay
Frost & Sullivan

"Microfluidics and nanochemistry are set to bring in great advances in the field of drug discovery."

treatments through autologous cells reprogrammed to meet the therapeutic need of the donor patient, will likely both become available," Mr. Aldrich says.

Jason Hwang, M.D., executive director, healthcare, at Innosight Institute, says in the near term, oncology will remain among the most active areas of development, as diagnostics redefine cancer pathways and make new targeted treatments possible.

"Longer term, widespread diseases with poorly understood mechanisms will undergo more precise definition and treatment, transforming, and likely renaming, diseases such as diabetes, hypertension, and depression," he says.

Dr. Hwang says profit margins that have long favored therapeutics over diagnostics will begin to shift, as "precision" diagnostics enable new, targeted therapies to emerge. The R&D business models that survive this transition will be those that have built processes and partnerships to develop therapeutics in conjunction with companion diagnostics.

"As diagnostics begin to drive more value in the therapeutics industry by making targeted drugs possible, they will decommoditize,"

Sound Bites From The Field

PHARMAVOICE ASKED INDUSTRY EXPERTS TO IDENTIFY TRENDS IN DRUG DISCOVERY INNOVATION AND TO PREDICT WHAT THE FUTURE HOLDS FOR INNOVATIVE MEDICINES.

KATRINE BOSLEY is CEO, Avila Therapeutics Inc., which focuses on design and development of covalent drugs to achieve best-in-class outcomes that cannot be achieved through traditional chemistries. For more information, visit avilatx.com.

"While drug classes such as biologics and nucleotide-based medicines will continue to develop, small-molecule therapeutics will remain the backbone of our pharmacopeia.

And despite the magnitude of our industry's aggregate efforts in medicinal chemistry over the decades, it's important to recognize that there are deep possibilities for novel, disruptive chemistry-based approaches to create best-in-class drugs that cannot be achieved with traditional medchem approaches.

As our understanding of disease targets and biology becomes ever-more subtle, we need innovative kinds of chemistries to harness these insights and deliver molecules that meet our goals to create first-in-class and best-in-class drugs. New chemistry technologies are emerging to exploit previously unexplored chemical territory, resulting in product candidates that can deliver unique product profiles."

MICHAEL N. CHANG, PH.D., is President and CEO of Optimer Pharmaceuticals Inc., a biopharmaceutical company focused on discovering, developing, and commercializing innovative anti-infective products to treat serious infections. For more information, visit optimerpharma.com.

"To address the issue of so-called dry pipelines, small biotechs will need to focus on collaborations to share profit and risk with late-stage compounds, since outright acquisitions are becoming stale. Resources can then be devoted to moving earlier-stage compounds forward to bolster the dry pipelines.

Big pharma companies will rely on purchasing later-stage, proven assets to reduce risk and fill in their own pipeline gaps."

KAREN FERRANTE, M.D., is Senior VP, Clinical, at Millennium: The Takeda Oncology Company, a biopharmaceutical company. For more information, visit millennium.com.

"Personalized medicine — targeting therapies for each patient — has the potential to be an important part of the future of drug development; biomarkers will be used to target select diseases and select patients."

ED SELLERS, M.D., PH.D., FRCPC, FACP, is VP, Early Stage, Kendle, a global clinical research organization providing the full range of early- to late-stage clinical development services. For more information, visit kendle.com.

"In general, data management will be minimally affected with the exception that trials may be smaller. Patient recruitment will change because the screening process will need to be applied to many who may not qualify for the trial.

Trial management may be complicated because patients at higher risk will be involved and may require more clinical supportive care. The issue of what to tell patients about their risk factor has a number of challenging ethical and practical issues."

STEPHEN M. SIMES is President and CEO, BioSante Pharmaceuticals, a specialty pharmaceutical company focused on developing products for female sexual health, menopause, contraception, and male testosterone deficiency. For more information, visit biosantepharma.com.

"Stringent regulatory scenarios and increased R&D spending are major factors restraining innovation. Efforts are ongoing to introduce transatlantic integration of regulatory processes.

In addition, the pharmaceutical industry is resorting to changing its business models to reduce the innovation costs and to offset the loss due to patent expirations."

TOM STEINKE is CEO, Minnow Medical Inc., a development-stage company focused on products for the treatment of peripheral artery disease. For more information, visit minnowmedical.com.

"Limited investment capital coupled with exuberant regulatory oversight will impede medical innovation substantially. There will be an increasing gap between university research and commercial sales.

That gap is expensive, requiring substantial capital, greater than can be provided by angel investors. I believe the focus will move away from medical to other more lucrative areas, such as energy, sadly resulting in a reduction in the quality of life for the aging population."

DANIEL ZURR, PH.D., is CEO and President of Quark Pharmaceuticals Inc., a development-stage company that discovers and develops novel RNA interference-based therapeutics. For more information, visit quarkpharma.com.

"Big pharma is usually mentioned in the same sentence with the term 'dry pipeline.' They could benefit by providing more funding to smaller and more nimble companies and academia where inventive research mainly takes place. For small-cap companies, the key will be more strategic partnerships and networking. Governments could do more to fund research, make the regulatory path less burdensome, and ensure healthcare is more affordable. Research into new technologies exists but most of the time it struggles to thrive."

he says. "That is, rather than being a nameless component in the therapeutics delivery process, they have the potential to become the 'Intel Inside' for their paired drugs."

According to Scott Treiber, Ph.D., MBA, executive VP, clinical development solutions, at inVentiv Clinical Solutions, personalized medicine — the shift from one-size-fits-all, evidence-based medicine to tailoring therapeutics using an individual's genetic makeup or specific metabolic response — will have a dramatic impact on the way drugs/biologics are developed and, ultimately, on the way medicine is practiced.

"The goal of this translational medicine, to achieve optimal response to drugs/biologics for a broader patient population, will require changes to policy, training, and economic models as we know them now," he says.

Simon Higginbotham, senior VP and chief marketing officer at Kendle, says the next great advancement will be in the area of genomic markers research.

"Genomic markers will eventually make possible the personalized medicine that the market has anticipated for so long," he says. "Pharma companies will develop whole families of drugs rather than traditional blockbusters, and genomic markers will then determine which variant of a particular drug will best treat individual patients."

Julian Adams, Ph.D., president of research and development and chief scientific officer at Infinity Pharmaceuticals, says in oncology it will be exciting to see if companies can tap into the advances around understanding tumors at the level of their molecular pathology.

"If we can improve our technology to identify and monitor relevant biomarkers, I think we could change the direction of R&D over the next five years," he says. "We need to understand the genetic transformation that occurs in different types of tumors and cancers. If we can do that, we can explore this exciting — but very complicated — area of research to develop a new wave of therapies that might provide the greatest benefit to patients."

Mr. Higginbotham says oncology, diabetes, and CNS areas will continue to be strong development areas, and there will likely be a dramatic increase in research into diseases of aging.

"As baby boomers continue to age, demand for these products will grow steadily," he says.

Michael Chang, Ph.D., president and CEO of Optimer Pharmaceuticals, says with the rise of emerging drug-resistant bacteria strains and diminishing efficacy of marketed treatments, antibiotics have re-emerged as a focal point for drug development.

"Pharma largely pulled out of anti-infective development over the last decade, citing saturation of the market, but with the emergence of superbugs like MRSA and Clostridium difficile, the medical community is now shorthanded with effective antibiotics," he says. "With pharma hungry to fill pipelines and biotech pushing new compounds into late-stage development, anti-infectives are one of the hottest market segments to watch and will continue to be in the coming year. In the next five years, it seems that cancer, anti-infectives, and aging-related diseases will be of particular interest."

A narrow disease focus concerns Stephen Simes, president and CEO of BioSante Pharmaceuticals.

"Unfortunately, it seems that future development will be limited by the rather myopic view among some that oncology, diabetes, obesity, and cardiovascular medicine are the only projects worth funding," he says. "Women's healthcare product development is an area that has been ignored, other than for oncology products, and therefore women do not have the same choices and options that men have; treatment of their sexual functioning is a prime example."

Tim Bertram, Ph.D., senior VP, science and technology, at Tengion, says regenerative medicine is another frontier slated for growth.

"There are more than 100,000 Americans on the waiting list for donor organs, and many others undergo significant surgical procedures to address failing organs," he says. "Regenerative medicine could truly revolutionize how we treat organ failure throughout the body by creating new organs and tissues." ✦

PharmaVOICE welcomes comments about this article. E-mail us at feedback@pharmavoice.com.

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