R&D Innovation: The Key to *Long-Term* SUCCESS

Pharmaceutical companies arguably put innovation on the back burner over the past decade as they looked to trim costs and meet shareholder expectations. Going forward, experts predict that by shifting the focus to biologics, genomic advances, personalized medicine, and specialty markets, innovation will have a multiplier effect on the industry.

When the scientific discoveries and pipeline-building process of many successful biotechs are layered together, the results are likely to be dramatic. Experts predict there will be a number of game-changing product candidates in development in the next decade.

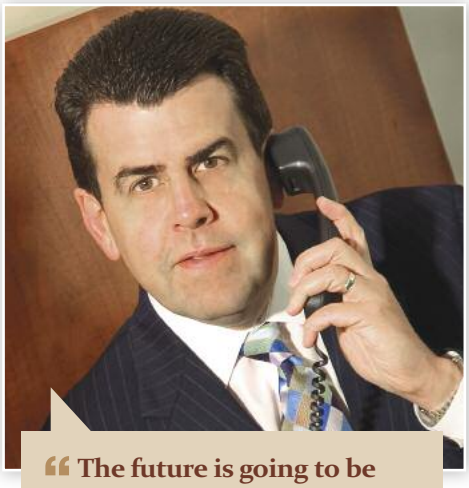
"We view ourselves as part of the 'multiplier effect' of innovation emerging from biotech companies," says Doug Ringler, V.MD., CEO of Tolerx, a biopharmaceutical company developing novel therapies to treat autoimmune diseases and cancer by normalizing the immune response. "Within the short-time span of a decade, a biotech company, such as Tolerx, can bring its first drug to near-commercial reality as a potential breakthrough for patients, as well as develop a pipeline of additional drug candidates based on a novel approach."

The biggest paradigm shift over the last decade has been the move of biologics, particularly monoclonal antibodies, to the center of drug discovery and development at almost every major pharmaceutical company and for nearly all indications, says Bassil Dahiyat, CEO of Xencor, which engineers biotherapeutics.

"Biologics create a totally new set of opportunities to treat diseases that were not available in the small-molecule-only mindset of the past," he says.

Within the last 10 years, there has been incredible growth in the development and approval of biologics, particularly monoclonal antibodies (mAbs), for the treatment of cancer and autoimmune disease.

"The progress that has been made to efficiently engineer and manufacture mAbs will help enable the development of targeted therapies for additional applications," says Brad Thompson, CEO of Oncolytics Biotech, a biotechnology company. "There's no question that personalized medicine will be the biggest market shaper in the next 10 years. The industry continues to advance its understanding of the genetic basis of many diseases, with the ultimate goal of developing treatments that can treat individual patients more effectively. Oncolytics has several trials that are looking specifically at patients pre-screened for KRAS- and EGFR-activated tumors and are working hard to realize the benefits personalized medicine may offer in the future."



> "The future is going to be based on personalized medicines based on each person's unique DNA and disease."
>
> **JOHN CLINE** / unithink

> "In the next 10 years, the industry will continue to transform through the identification and implementation of new development models."
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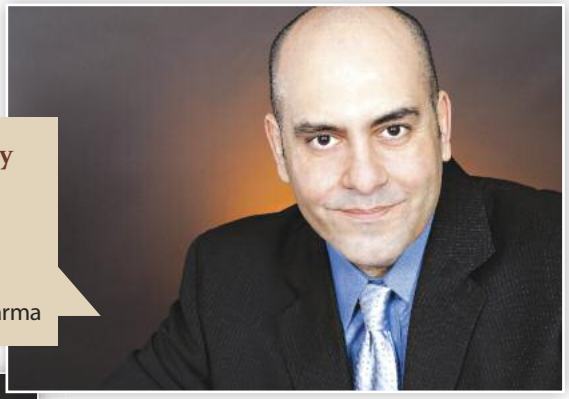
> Parexel International



> "RNA as a novel and widely applicable drug target has transformative medical potential."
>
> **CHRIS GARABEDIAN** / AVI BioPharma

> "While the days of mass-market blockbusters may be over, this term will be redefined, and the new blockbusters will be specialized therapies."
>
> **KEN RIBOTSKY** / The Core Nation

Simon Moroney, Ph.D., CEO of MorphoSys, a biotechnology company developing therapeutic antibodies, predicts that monoclonal antibodies will remain one of the leading drug categories within the biotech and pharmaceutical industry.

"Monoclonal antibodies can be used as tools for diagnosis and treatment of a variety of different diseases," he says. "Sophisticated technologies that enable a new generation of antibodies with very precise properties, allied with a deeper understanding of target biology, will produce drugs that will proceed through development more quickly and with higher success probability. Patients will benefit from therapies that are tailored to their needs."

Innovation at the Molecular Level

Dr. Ringler has no doubt that the biggest change in the next 10 years will be how much closer companies will get to creating "cures" for many diseases, as opposed to treating the consequences of the diseases.

"With the innovations coming from biotech and therapies that identify, target, and repair the root cause of the disorders, the drugs emerging in the next decade should be much closer to cures because they will lead to disease remission, relapse prevention, and correcting or halting disease processes," he says.

There is little dispute that the public release of a rough draft of the human genome in 2000 was the single biggest game changer within the first decade of the 21st century for the life-sciences industry, says Michael Parks, executive VP, public relations and marketing communications, at Vox Medica, a healthcare communications agency.

"This achievement spearheaded by The Human Genome Project and Celera Genomics gave new meaning to the possibilities of preventive medicine, where initiating true prevention could begin before a child is even born through genetic testing."

One genomic area that holds promise, according to several thought leaders, is RNA.

"The rapid progress in understanding RNA as a therapeutic target and innovative chemistries with the potential to deliver high-potency, low-toxicity, and easily adaptable drugs, point to RNA as a novel and widely applicable drug target with transformative medical potential," says Chris Garabedian, president and CEO of AVI BioPharma, a biopharmaceutical company discovering and developing RNA-based therapeutics for rare and infectious diseases. "Directly attacking RNA, the carrier of genetic information, rather than enzymes or receptors like many traditional therapeutics, makes heretofore undruggable targets accessible."

By a process of alternative RNA splicing, close to 90% of genes produce several different proteins from the same gene, he explains.

"Manipulation of alternative RNA splicing can not only reduce or eliminate expression of undesirable proteins, like other RNA-based technologies, such as siRNA and antisense, but also restore production of desired proteins," Mr. Garabedian says. "This

Biotech Predictions for 2011

» **Biotech and the Capital Markets:** The biotech industry did benefit from the return of investor confidence in the second half of 2010, with the Burrill Biotech Select Index outperforming the Dow Jones Industrial Average on an annual basis. Expect to see the biotech industry continue to outperform the general markets as the financing environment continues to improve in 2011.

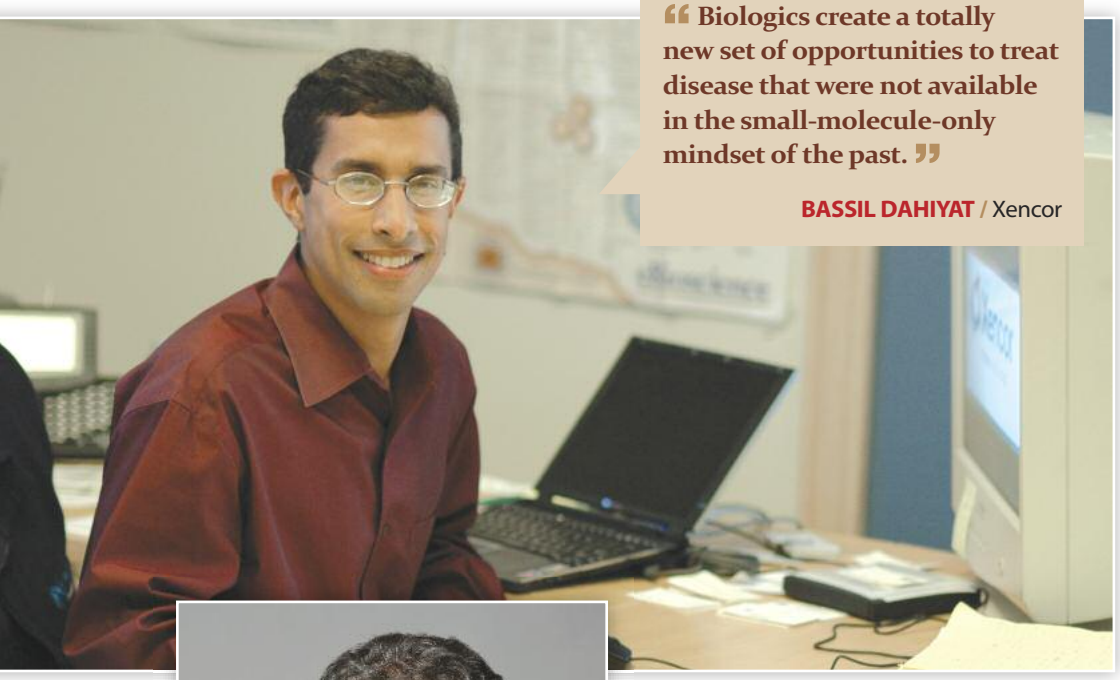
» **Biotech IPOs:** The biotech IPO window will remain open despite the fact that the 17 new biotech issues that debuted on the U.S. market in 2010 were plagued by lackluster receptions (selling fewer shares below the pricing range) and their average annual market performance was down by 13%. By the end of 2011, at least 25 biotech IPOs, possibly more, in the U.S. will be completed.

» **Capital:** The industry has achieved a steady state in terms of financing, raising about $15 billion annually. This situation will continue in 2011. The industry's collective market cap will also remain at its present $360 billion level as market value growth will be offset by acquisitions.

» **Biosimilars:** Healthcare reform carries provisions instructing the FDA to create a pathway for biosimilars. Expect to see both biotech and pharma companies take an interest in the drafting of new regulations governing the development of biosimilars.

» **Converging technologies impact healthcare:** Expect to see a greater emphasis on prevention and wellness, as well as a greater understanding of human genomics and the advent of molecular diagnostics, and the convergence of information, wireless, and medical technology promise to make personalized medicine an ever-present reality in the way doctors and patients approach healthcare.

Source: Burrill & Co.
For more information, visit burrillandco.com.



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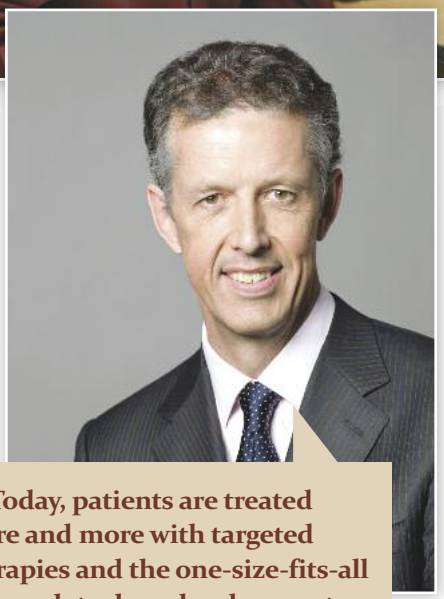


> "Today, patients are treated more and more with targeted therapies and the one-size-fits-all approach to drug development has become a thing of the past."
>
> **DR. SIMON MORONEY** / MorphoSys

technology is particularly attractive for the treatment of rare diseases, such as Duchenne muscular dystrophy where repair of defective RNA and restoration of missing dystrophin (protein) are essential for clinical benefit."

The discovery of RNAi has had a huge impact, agrees Helge Bastian, VP, research biotech/global marketing, business development and strategy for Sigma Life Science, and has provided a better understanding of biology and shaping the landscape of the industry. Sigma Life Science offers an array of biologically rich products and reagents that researchers use in scientific investigation.

"In addition, there have been other major developments over the course of the past 10 years," she says. "Carving out ZFN and iPS technology has been exciting. Both are just starting to have an impact. ZFN- or gene-editing as well as iPS technology are the game-changers. These, plus a highly sensitive and multiplex protein detection system will reshape the research market and allow us to see things that haven't been detectable before. In addition, I believe that companion diagnostics and the personalized medicine sector will eventually find a prominent place in the market."

Zinc finger nucleases (ZFNs) are a class of engineered DNA-binding proteins that facilitate targeted editing of the genome by creating double-strand breaks in DNA at user-specified locations. Double-strand breaks are important for site-specific mutagenesis in that they stimulate the cell's natural DNA-repair processes.

David Smoller, president of research biotech at Sigma Life Science, says while in the last 10 years the sequencing of the human genome made a big impact on the industry with RNAi driving the understanding of this genome, adult stem cells with the power of ZFNs will change the future of the industry and the understanding of biology.

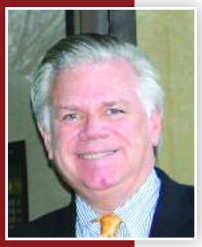
"At the same time I would not underestimate the power of the genetically modified rat using ZFNs," he adds. "The rat could help revolutionize how we evaluate drugs for safety and drug-drug interactions and open doors to understanding the brain and the diseases that effect the brain, such as Alzheimer's, Parkinsons, etc. Finally, nextgen sequencing will have a profound effect on personalizing medicine."

Experts agree that biopharmaceuticals will be the dominant force in terms of market growth in healthcare.

"Over the next 10 years, the creation and advancement of new technology platforms will allow for the rapid, methodical, and systematic study of new biologic drug candidates," says Bill Newell, CEO of Sutro Biopharma, a biopharmaceutical company. "These novel technologies will circumvent many of the current limitations of biologic

BACK TO THE FUTURE

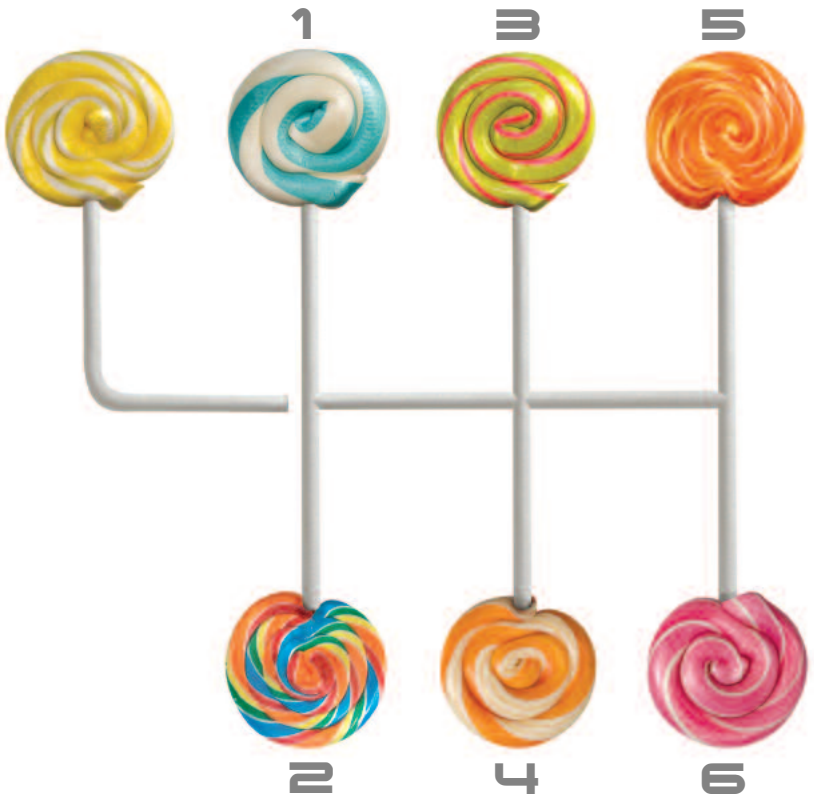
THE FUTURE OF BIOTECH



TERRENCE TORMEY
CEO and President, Prevention Pharmaceuticals

» **THEN (2001):** [Biotech] companies looking to do their own marketing and sales recognize they don't have the internal resources necessary, so they're going to outsource those functions — everything from the marketing, to the selling, to the ancillary services.

» **NOW:** With few exceptions, biotech companies have not lived up to their potential in product commercialization. Biotech CEOs are more interested in raising monies and quickly exiting. As a result, the very day a major pharma company expresses an interest in their NDE, they say, "sold." I had once believed that these CEOs and their investors would come to understand that by outsourcing sales, marketing, manufacturing, etc., they could hold onto the NDE and enjoy perhaps hundreds of millions of dollars of revenue for their company. Instead, these otherwise extraordinarily bright M.D.s/Ph.D.s. are foregoing their advantage. Major pharmaceutical companies, on the other hand, faced with product pipelines that are as dry as a west Texas oil well, are quickly buying up biotech companies and/or their products.



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> "Almost all new agents under development in cancer have a specific target identified on a molecular basis and therefore these agents, even in the earliest trials, are much more likely to clinically benefit the patient."
>
> **DR. STEPHEN JONES** / US Oncology

> "The continued adoption of molecular medicine is poised to completely reshape every part of the healthcare system."
>
> **MARCIA KEAN** / Feinstein Kean Healthcare

> "Science has moved rapidly, and R&D scientists, regulators, and providers are now chasing treatments and cures for a variety of diseases with a rising bar."
>
> **MICHAEL KLEINROCK** / IMS Health

drug discovery and development and will enable the realization of novel therapeutics providing important new treatment modalities that are not realizable today."

The continued adoption of molecular medicine — facilitated by informatics systems that enable collection, aggregation, analysis, and dissemination of data — is poised to completely reshape every part of the healthcare system, from research and clinical development, to how products are reimbursed, to the practice of medicine itself, says Marcia Kean, CEO of Feinstein Kean Healthcare, a communications and business consulting firm.

"For biomedical innovators, the challenge will be to use data/knowledge more efficiently — and pre-competitively — to develop new products that prevent and change the course of disease, leverage companion diagnostics to identify the appropriate patient population for their drugs, and engage with an increasingly data-empowered group of consumers to accelerate and improve clinical development."

One of the big game changers in the last 10 years has been the discovery and development of Novartis' breakthrough cancer therapy Gleevec. The drug was discovered using an understanding of molecular pathways, and it works by specifically targeting proteins in cancer cells, but leaves healthy cells unharmed.

"This has been a huge breakthrough for cancer therapy as Gleevec has an 86% overall survival rate at seven years, the longest observed overall survival in blood cancer," says Joe Jimenez, CEO of Novartis, a global healthcare company. "Gleevec's discovery has also led us to further innovations; for example we developed Tasigna, which studies show performs better than Gleevec in slowing disease progression for newly diagnosed chronic myeloid leukemia patients.

"I see this as a real turning point for the industry because it literally transformed chronic myeloid leukemia; patients had a life expectancy of three to five years once diagnosed in the chronic phase, before Gleevec," Mr. Jimenez continues. "Now it may be possible for CML patients to expect to have a normal life expectancy."

While not a biologic, the introduction of thalidomide in the last decade for the treatment of multiple myeloma was another game changer, according to Brian G.M. Durie, M.D., co-founder and chairman of the International Myeloma Foundation and co-chair Myeloma Committee, SWOG. He is also the medical director of Aptium Oncology Myeloma Consortium (AMyC) Aptium Oncology, which provides solutions for oncology care.

Thalidomide was introduced as a sedative drug in the late 1950s. In 1961, it was withdrawn because of teratogenicity and neuropathy. Today, there is a growing clinical interest in thalidomide, and it has been re-introduced as an immunomodulatory agent used, in combination with dexamethasone, primarily to treat multiple myeloma.

"Thalidomide opened the doors to a dramatic new paradigm of treatment, demonstrating that non-cytotoxic approaches could work in the treatment of multiple myeloma and related blood cancers," Dr. Durie says. "This paved the way for other novel therapies, the thalidomide-relatives Revlimid and pomalidomide, and the proteasome inhibitors Velcade and more recently carfilzomib. This sea-change in treatment provides extended remissions with improved quality of life for patients. Ten years from now we expect to see pharmaceutical treatments replacing stem cell transplants, and

> "Genetic tests and profiling are improving the ability of physicians to match patients with the most beneficial treatments."
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> **DAVE FISHMAN** / Snowfish

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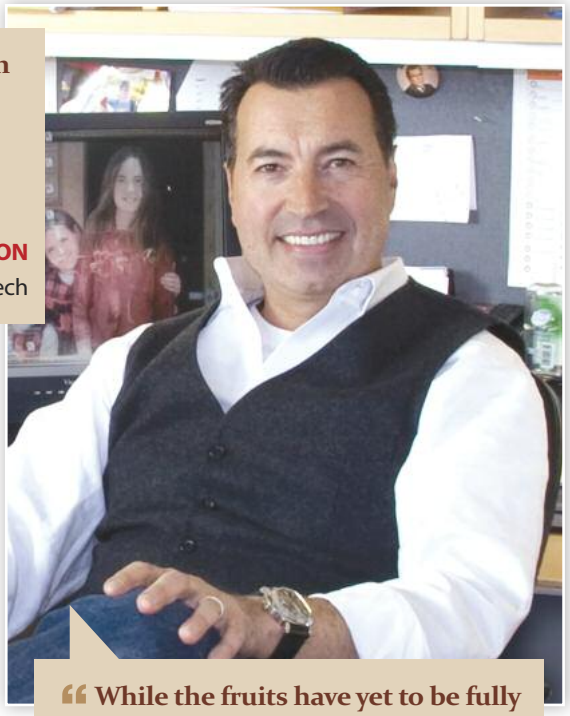

> "While the fruits have yet to be fully harvested, the mapping of the genome has paved the way for a new world of genetic testing and personalized medicine."
>
> **MATT GIEGERICH** / Ogilvy CommonHealth

we expect to identify at least the first cohort of patients who can be cured with a combination or sequence of pharmaceuticals."

According to Patrick Flochel, Ernst & Young's Life Sciences Leader for EMEIA, what will separate winners from losers in the next 10 years will still be the scientific excellence that companies will be able to emulate.

"However, for science to fuel the commercial success of life-sciences companies, companies will need to be able to develop the agility to run successfully different business models simultaneously to deliver the best health outcome to different stakeholders in different markets," he says.

Julian Parreño, senior account director at Datacore Marketing, which combines data-driven intelligence with one-to-one marketing innovation, says creating more streamlined R&D organizations, including de-risking initiatives for clinical candidates entering Phase III, and live licenses, will be critical to discovering and developing the successful compounds of the future.

"In an increasingly cost-focused environment, demonstrating the innovativeness of new products will also be a key factor," he explains. "The definition of innovation varies among multiple stakeholders — researchers, investors, payers, regulators, providers, manufacturers, and patients — but in the end, new products priced in balance with the value delivered equals innovation. The industry needs to focus on investing in developing medicines the market will buy."

Rob Likoff, co-CEO of Group DCA, an interactive agency, adds that innovation in the face of increasing government intervention and oversight will be key to the industry's success in the next decade.

"The market can't support 10 versions of the same drug any more," he says. "Companies that innovate in the lab, in operational efficiencies, in communication outreach will be the survivors. The danger communicators face is getting caught up in the technology and overlooking the true driver of this transformation: the healthcare profession's need to receive content of value. Content needs to be the focus. Companies that understand this and that tap the efficiencies of multichannel communications, effectively pairing personal with digital outreach, will win."

The Genome

Ten years ago, the excitement of unlocking of the human genome had many thinking that personalized medicine couldn't be far behind. Then reality set in; it has proven to be much more difficult to take the discoveries stemming from the unraveled genome in the lab and translate them into therapies. There is excitement, however, again, as many believe the industry is just one generation of medicine away from taking a huge leap forward.

"The mapping of the human genome, in my opinion, is the most significant advancement in healthcare," says Michael Parisi, managing partner at Ogilvy CommonHealth Worldwide, a healthcare communications network. "Originally funded by the U.S. Department of Energy, this initiative was undertaken to gain critical knowledge about humans that would enable us to better understand the causes of disease and, more importantly, help develop medications to treat disease. It was truly the beginning of personalized medicine. And the promise of personalized medicine and the development and use of diagnostic tests to better

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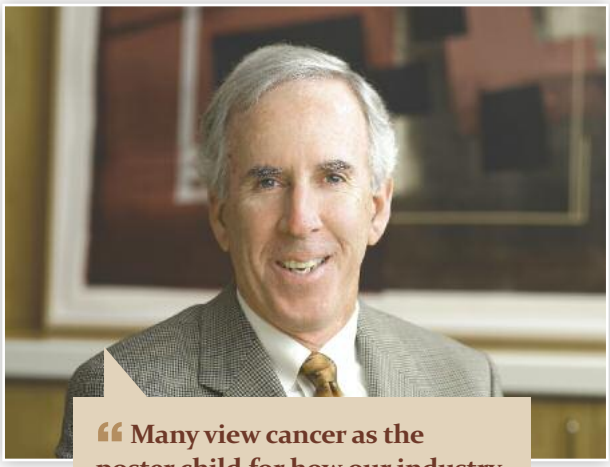

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>
> **JOE JIMENEZ** / Novartis

characterize diseases and select treatment are the game changers of the future. I hope we'll see the routine use of companion diagnostics and targeted therapies change the face of serious illnesses around the world."

Jed Beitler, chairman and CEO Worldwide of Sudler & Hennessey, a global healthcare marketing and communications organization, agrees that the mapping of the human genome has been one of the biggest game-changers in the life-sciences field.

"Not only has it opened the doors to better understanding of diseases and their origins, but it has also led to the development of better diagnostic tools and therapies to more precisely detect and attack these disorders," he says.

Matt Giegerich, chairman and CEO, Ogilvy CommonHealth Worldwide, a healthcare communications network, says while the fruits of this mind-boggling project have yet to be fully harvested, it has paved the way for a new world of genetic testing and more effective and efficient personalized medicine.

Rather than the traditional organ-based approach to disease and the traditional one-size-fits-all approach to developing therapeutics, academe and industry have shifted their focus to unraveling the underlying mechanisms of disease at the molecular level and customizing medicines to molecular subgroups of patients, Ms. Kean says.

"Today, almost all life-sciences companies are using information on genetic variation and its effects to delineate disease more precisely and to enhance the safety and efficacy of new treatments," she says. "And, more and more academic medical centers and other pioneering healthcare providers are reshaping their infrastructure to accommodate these changes."

One research community that is eager to capitalize on the therapeutic potential of genomic/targeted therapies is oncology.

"Many view cancer as the poster child for how our industry has made game-changing innovations to treat disease," says Dan Junius, president and CEO of ImmunoGen, which develops targeted anticancer therapeutics. "As of 10 years ago, we became armed with more in-depth knowledge of cancer at a biochemical and cellular level, and this has led to targeted therapies for specific types and stages of cancers, rather than a one-size-fits-all approach. After the progress of the last decade, there are now more than 20 targeted cancer therapies on the market, with a pipeline of more than 100 in clinical studies as potential new cancer drugs."

"Almost all new agents under development in cancer have a specific target identified on a molecular basis and therefore these agents, even in the earliest trials, are much more likely to clinically benefit the patient," says Stephen Jones, M.D., medical director and co-chair of the breast cancer committee with U.S. oncology research at US Oncology, a division of McKesson Corp. "This is a far cry from 10 years ago when new agents rarely helped anyone. Despite the promise of this approach — and we all recognize that this is the future — there are relatively few approved agents based on molecular mechanisms or targets."

One targeted therapeutic that has changed the natural history of one type of breast cancer — HER2 positive disease, 20% of all invasive breast cancers — is Herceptin, which was developed as a monoclonal antibody to the oncogene HER2.

"The routine use of this treatment has markedly and forever favorably affected the natural history of this type of formerly deadly breast cancer," Dr. Jones says. "The oncology community welcomes the targeted approach to treatment in years to come."

Biomarkers and targeted therapies are clearly the biggest game changers and industry drivers of the past decade and for the near future, says Kevin Coker, VP of U.S. oncology research at US Oncology, a division of McKesson Corp.

"By targeting therapies to individual genetic markers, scientists can more precisely target unique cancer types," he says. "This new information leads to better trials and helps guide expectations on product life-cycle management. As the course of disease is better understood, it becomes more predictable, which leads to better outcomes for the patient and, ultimately, lower costs to the healthcare infrastructure. Although personalized medicine is still in its early days, this all holds great promise."

No question about it, personalized healthcare will be the game changer for the industry over the next 10 years, says Scott Cotherman, CEO of CAHG, an integrated marketing communications network.

"The application of genomic information to diagnostics and therapeutics is one of the most significant developments in health and wellness," he says. "The promise of personalized healthcare — driven by increased understanding of the human genome, advances in

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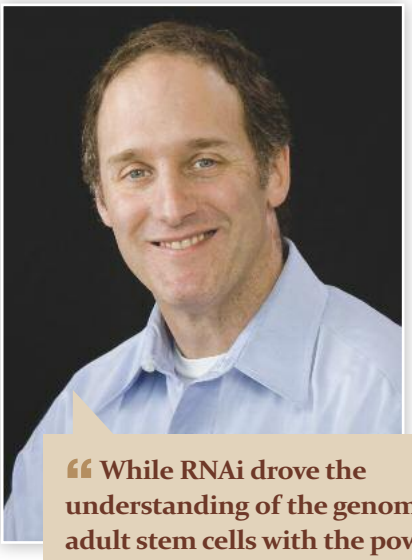
>
> **DR. BRIAN DURIE** / International Myeloma Foundation

> "Innovation that improves overall efficacy and patient outcomes, while decreasing healthcare expenditures, will clearly drive the shape of the market over the next 10 years."
>
> **MICHAEL PARKS** / Vox Medica

> "Companies will spend more time thinking about how best to leverage informatics and companion diagnostics to improve the economic viability of niche drugs."
>
> **DR. STEPHEN DECHERNEY**
> MedAssurant



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>
> **DAVID SMOLLER** / Sigma Life Science

molecular diagnostics, and targeted therapies — is exciting for the industry, as it holds the potential to enhance wellness, provide better treatment outcomes, and improve overall quality of care.

"These opportunities, however, will bring challenges for the industry as it works through issues such as identifying the right biomarkers; conducting clinical trials with smaller patient populations; managing co-approval pathways of companion diagnostic/therapeutic offerings; and optimizing value propositions in the face of more sophisticated payers and benefit managers," Mr. Cotherman continues.

If the future is going to be based on personalized medicines based on each person's unique DNA and disease, then the safety and testing around these drugs will change the face of research and development, contends John Cline, CEO of unithink, an electronic clinical research organization.

"The next 10 years in the e-clinical space will be about figuring out how to apply technology to allow for the rapid design and testing of highly unique individualized medicines," he says. "The second challenge will be how to manufacture those unique medicines in a cost-effective method."

Personalized Medicine

The amazing breakthroughs witnessed over the past 10 years have been incredible; the decoding of the human genome, which opened the door for personalized medicine, and the proliferation of companies that comprise the biotech industry have been true game changers, says JeanMarie Markham, CEO of Clinlogix, a next-generation CRO.

"Yet we are only peering into the possibilities of tomorrow," she says. "It is humbling when one takes in the genius of what has been accomplished. Despite the challenges, the industry's ability to take these head on, adapt, and focus on the future and envision what can be paramount to success; it's this resiliency, determination, and freedom that fuel our collective imagination and feed innovation."

Novartis' Mr. Jimenez says the key for the future will be developing algorithms that will allow us to unlock information in the millions of terabytes of genetic data available and to create new molecular tests that will play an integral role in our targeted therapy approach.

"Advances in biology and bioinformatics are already enabling unprecedented analysis of human genes and proteins, and we are starting to genetically identify which patients will respond positively to particular therapies," he says. "At Novartis, we recognize the possibilities for this area and have established a dedicated Molecular Diagnostics Unit, focused on matching the right patient with the right drug. We have a rapidly expanding portfolio of diagnostic programs and are aiming for multiple launches over the next few years."

MorphoSys' Dr. Moroney says a clearer understanding of human diseases has paved the way for completely new treatment options and today, patients are treated more and more with targeted therapies and the one-size-fits-all approach to drug development has become a thing of the past.

"Understanding the differences between diseased and healthy cells has furthered the development of groundbreaking targeted

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>
> **JEANMARIE MARKHAM** / Clinlogix

therapies, such as monoclonal antibodies, most notably in cancer," he says. "Today, several mAbs are blockbuster drugs."

With the emerging niche-buster paradigm, focused on therapies that address specific disease areas, commercial success depends on moving a larger number of compounds through the discovery and development gates into the market, says Josef von Rickenbach, chairman and CEO of Parexel International, a global biopharmaceutical services provider.

"This will take a different kind of R&D strategy, and there will continue to be an increasing focus on identifying winning compounds and validating those compounds through to proof-of-concept," he continues. "Overall, in the next 10 years the industry will continue to transform through the identification and implementation of new development models and ways to achieve greater efficiencies in the R&D process."

Dave Fishman, president of Snowfish, which provides commercial insights to healthcare, life-sciences, and biotechnology companies, believes that the days of multi-billion dollar products with near universal applicability are drawing to a close.

"Physicians have long-recognized that any given drug may in fact be more effective in certain patient types than in others," he explains. "Looking ahead, genetic tests and profiling are improving the ability of physicians to match patients with the most beneficial treatments. While targeted therapies will significantly improve the approach to patient care, the industry will be challenged to ensure that the optimal ROI is obtained given the smaller markets for these products. Companies will need to have a greater understanding of market segments and the opportunity they represent in order to align investment with expected returns."

Stephen DeCherney, M.D., senior VP of pharmaceutical and life sciences at MedAssurant, a medical informatics solutions provider, agrees that without the blockbuster, companies are and will pursue niche products that are specifically targeted to patients of different genotypes and phenotypes.

"Employing the model of a rifle — personalized medicine — versus a shotgun — blockbuster drugs — over the next few years, companies will spend more time thinking about how best to leverage informatics and companion diagnostics to improve the economic viability of niche drugs," he says. "The challenge remains that the technical, regulatory, and economic models for developing, approving, and profiting from personalized medicine have yet to be fully developed. However, this provides ample opportunity for first-mover advantage."

> “Going forward, specialty pharma is the road to innovation that can change the human condition.”
>
> **GIL BASHE** / Makovsky + Company

> “I hope we’ll see the routine use of companion diagnostics and targeted therapies change the face of serious illnesses around the world.”
>
> **MICHAEL PARISI** / Ogilvy CommonHealth

> “The business model for developing and commercializing therapies will, and must, look very different in the future.”
>
> **TIM SHANNON** / Canaan Partners

> “We view ourselves as part of the ‘multiplier effect’ of innovation emerging from biotech companies.”
>
> **DR. DOUG RINGLER** / Tolerx

Mr. Cotherman says the industry faces marketing and sales challenges as physicians — and their patients — are woefully uneducated and unfamiliar with genomics-based medicine.

“Marketing communications agencies that possess deep and broad knowledge of this emerging area and its stakeholders will be well-positioned to help their clients thrive in this new era of healthcare,” he says.

Mr. Beitler say because much of the healthcare industry is now focusing on specialty drugs that target smaller medical needs-based populations, there will be dramatic changes in marketing and promotion as companies will no longer have the need to market more broadly.

The next 10 years will provide even deeper insights into the biological underpinnings of disease leading to novel drugs that are more and more targeted at these fundamental sources of disease.

“Both biotech and pharma companies are well-positioned to convert this deep and focused translation of science and an expanding experience base into ever-more-targeted drugs that open the possibilities for significant improvements for patients across a wide range of diseases,” ImmunoGen’s Mr. Junius says.

Jay Bigelow, CEO of MicroMass Communications, a behavior science-based communications agency, says as the FDA starts to approve treatments for patients with acceptable genetic profiles, the industry will reinvest in new drug development and this will reopen the market to drugs that were previously denied.

“The life-sciences industry will respond with new business models that are built on partnerships and hybrid relationships with their managed care, physicians, and patient counterparts,” he says. “This business model will focus on patient outcomes, an emerging role for medical liaisons and transparent information sharing.”

Mr. Parks says innovation that improves overall efficacy and patient outcomes, while decreasing healthcare expenditures, will clearly drive the shape of the market over the next 10 years.

“As companies look to fill the estimated gap of $16 billion in annual revenue over the next five years alone, there will be far more collaborations between companies that can help drive innovation that adequately addresses patient needs, while finding the clinical and economic edge required to compete in an increasingly more complex and demanding payer environment,” he says.

The growing interest in and dedication to developing personalized treatments — documented by the recent Tufts Center for the Study of Drug Development study on the topic — has the most potential to transform healthcare, says Edward Abrahams, Ph.D., president of the Personalized Medicine Coalition, which brings together people with a commitment to personalized medicine.

“The industry’s commitment to personalized medicine will improve treatments in the next decade, especially if the FDA and CMS send the right signals to encourage greater investment in the development of safer, more efficacious drugs,” Dr. Abrahams says.

Despite a decade of critical challenges — safety concerns, quality problems, compliance failures, and pipeline difficulties — the industry is now truly research-based and driven by new knowledge of the human genome, molecular mechanisms of disease, treatment regimens, and the emerging power of personalized medicines, says James Macdonell, VP of Patni Americas, a global IT services company.

“These advancements allow us to develop and deliver products with unprecedented knowledge and control of quality, efficacy, and

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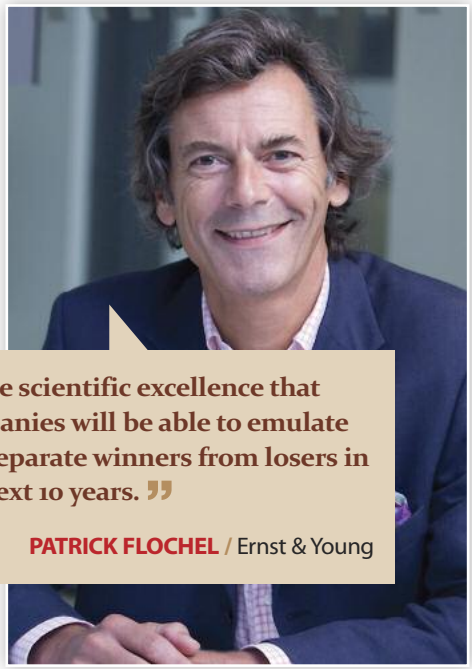
> "Biomarkers and targeted therapies are clearly the biggest game changers and industry drivers of the past decade and for the near future."
>
> **KEVIN COKER** / US Oncology



> "The scientific excellence that companies will be able to emulate will separate winners from losers in the next 10 years."
>
> **PATRICK FLOCHEL** / Ernst & Young

safety for targeted populations," he says. "Even as first treatments emerge, the critical issue of economic feasibility of personalized medicine is proving positive. For example, monogenic neonatal diabetes is now being successfully treated by personalized medicine, in a cost-effective manner, with improved outcomes."

Specialty Markets

In the last 10 years, the shift in innovation to more niche, orphan, and specialty pharmaceuticals has changed the market landscape from addressing unmet needs in large, chronic populations to focusing on unmet needs in smaller patient groups, says Michael Kleinrock, director of thought leadership at IMS Health, a market intelligence company.

"This has dramatically impacted the structure and delivery of healthcare and healthcare funding globally, and ultimately underlies the emergence and cementing of the patent cliff for large global pharmaceutical companies," Mr. Kleinrock says.

Chris Bogan, CEO of Best Practices, a research and consulting firm, agrees that over the decade, once-dominant primary-care business and treatment models have evolved toward specialty-care targeted therapeutics.

"This mega-shift has commenced slowly, but it is having profound consequences," he says. "The mass-market primary-care approach tried to produce blockbuster products promoted widely across general and specialty practices. Large sales forces, costly education and promotion programs, and large non-responder groups for whose treatments insurers reluctantly paid were all part of the landscape. Increasingly, specialty pharma models have blossomed. They are geared to deliver targeted therapeutics or treatments best-suited for specific conditions, specific sub-populations, and specific genotypes."

(Editors' Note: See the April 2011 issue for more on the specialty market.)

Ken Ribotsky, president and CEO of The Core Nation, a family of healthcare marketing and medical communications companies, says now that the human genome has been unraveled and that there are technologies available to influence what happens at the genetic level, big pharma companies are starting to invest more on therapies to treat orphan and rare diseases.

"There is a shift happening — from treating mass diseases to specialized diseases affecting smaller segments of the population," he says. "And there's tremendous potential for huge financial gains with these emerging therapies. The global orphan drug market is expected to reach $112.1 billion by 2014. While the days of mass-market blockbusters may be over, this term will be redefined, and the new blockbusters will be these specialized therapies."

> "The rise of personalized medicine will reopen the floodgates of innovation and bring new drugs to the market."
>
> **JAY BIGELOW** / MicroMass

During the most recent decade, the greatest paradigm shift in our industry, according to Stephen Wray, president and CEO of Cadient Group, an interactive marketing company, has been the shift in focus to specialty medicine.

"From the perspective of the industry's pipeline composition, the change in this direction has been monumental in reshaping the competitive landscape," he says. "As a result, traditional rules for engaging with healthcare practitioners, forging relationships with patients and caregivers, and gaining market access have been dramatically altered."

Tim Shannon, venture partner at Canaan Partners, a global venture capital firm, believes one of the biggest game changers of the past decade has been the emergence of rare diseases into the academic, regulatory, and commercial mainstream.

"This is evidenced by Sanofi-Aventis' efforts to acquire Genzyme, but also by the stated intentions of Pfizer, GSK, Novartis, and other big pharma companies to become active in these areas," he says. "These initiatives will set the stage for new commercial delivery models that will be much better positioned to support the accelerated emergence of personalized medicine products. The new models will look more like those of rare diseases than those of statins and blockbusters of the past. The most respected and valued companies in the next 10 years will be those that negotiate this transition successfully. The business model for developing and commercializing therapies will, and must, look very different in the future."

While the enactment of the Orphan Drug Act was more than 25 years ago, the biggest impact has been finding a workable financial model for treatments for rare diseases, says Gil Bashe, executive VP of Makovsky + Company, an independent global public relations, investor relations and branding consultancy.

"Balancing the high cost of discovery, development, and manufacturing with a price payers are willing to shoulder has opened the door to treatments — from Gleevec to Soliris — never imagined possible and lives are being reclaimed," Mr. Bashe says. "Going forward, specialty pharma is the road to innovation that can change the human condition." PV

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