

Trending 2018: Rare Diseases

With about 7,000 rare diseases affecting patients globally, many with no approved treatment, there is an enormous opportunity for companies to follow scientific developments to deliver much-needed treatments.

In recent years, a growing number of companies have entered the rare disease space, bolstered by regulatory incentives, such as the Orphan Drug Act, and scientific breakthroughs. While each rare disease has a relatively small patient population — defined in the United States as affecting fewer than 200,000 people — collectively the approximately 7,000 rare diseases affect more than 350 million men, women, and children globally.

Across the major markets of the United States and Europe there has been a marked increase in the number of applications and designations for orphan drug status. Evaluate analysts predict that orphan drugs will add \$95 billion in additional sales by 2022.

“Rare diseases offer a faster, cheaper, and therefore more lucrative — certainly in the short to medium term — path to commercialization than, say, common indications that would require studies involving thousands or even tens of thousands of patients,” says Yuval Cohen, Ph.D., CEO, Corbus Pharmaceuticals.

“The Orphan Drug Act, and its equivalent in other countries, has also been a tremendous incentive to focus on such indications.”

Pierre Laurin, B.Sc.Pharm., M.Sc., president and CEO of Prometic Life Sciences, agrees that legislation and the regulatory agencies have played a key role in creating an environment that is very favorable for companies that have the technologies and therapeutic solutions to address rare diseases.

“Different programs such as pediatric vouchers, orphan designation, market exclusivity, and special funding are some of the factors that have incentivized companies to pursue product development in the rare disease space,” he says. “This has refocused companies on finding treatments for patients facing rare and pediatric diseases — while revitalizing the biotech industry in the United States.”

According to Robert Blum, CEO of Cytokinetics, there are three major trends driving the growing interest in rare disease: research and innovation that is uncovering a better understanding of rare diseases and a more targeted approach to drug development; greater commitment by the FDA to accelerate the approval path; and patient advocacy that is helping to create greater awareness, voice, and public pressure for drug development buoyed by greater resources and enhanced communication channels.

Carter Keller, chief operating officer at GigaGen, says in recent years, smaller companies have focused more frequently on rare diseases where clinical success rates have proven to be higher,



clinical trials are smaller, and there is often a faster path to market.

“These start-up companies are conceived with a compelling rationale on how to treat a specific patient population and are able to attract investment dollars that years ago would have gone to companies focused on large chronic diseases,” he says.

Globally, many larger diseases are starting to be broken into sub populations with the increasing use of biomarkers and genetic information to screen patients for participation in clinical trials, which raises the success rate of these trials and can lead to better efficacy outcomes, Mr. Keller adds.

Igor Gonda, Ph.D., president and CEO of Aradigm Corp., says there is a fundamental, often forgotten difference between research into rare disease compared with chronic disease: the patients who participate in these trials take a much bigger risk than the companies conducting them.

“Pursuing development of treatments for rare chronic diseases is full of challenges not dissimilar from the attempts of explorers of which many fail before success is achieved,” he says. “Many of these patients spend a significant part of their life helping to find the cure for their disease — knowing well that they may never get any benefit themselves but their dedication could prevent their descendants from the disease ordeal. This is an extremely motivating factor for us.”

Aradigm has been developing a treatment — an inhaled antibiotic called Linhaliq — to



I would like to see companies that charge a lot for rare disease therapies introduce programs within their own organizations to keep pushing the envelope.

DR. LISA BOYETTE
Curable

help patients with a rare disease with unmet medical need, non-cystic fibrosis bronchiectasis (NCFBE).

“Our focus is on the subgroup of patients with chronic lung infections with a difficult microorganism, *Pseudomonas aeruginosa*, who have a particularly rapid decline in their quality of life, frequent hospitalizations, and high mortality,” Dr. Gonda says. “During this development program, we learned many lessons about designing appropriate clinical trials such as selection of the dosage regimens and endpoints.

“These lessons are likely to be useful in pursuing treatments in the wider population of patients who have chronic lung infections. In particular, there are millions of patients with chronic obstructive pulmonary diseases where these infections are also associated with poorer prognosis and premature death,” he continues.

Angi Robinson, executive director, strategic development, rare diseases and pediatrics at Premier Research, says while incentives that come with orphan drug development drive momentum, advantages including financial incentives such as tax credits, reduction in regulatory fees, and subsidies, marketing exclusivity, and access to tools and programs to accelerate drug approval are perhaps equally compelling.

“I’ve also seen a genuine desire from sponsors I’ve worked with to help these patients, along with a more classical scientific drive to understand these very rare diseases and formulate the science or technology that overcomes them,” Ms. Robinson says. “The science and the potential in rare disease are cutting edge and very exciting, and this is where drug makers want to be.”

Howard Mayer, M.D., senior VP and ad-interim head, global research and development, Shire, says rare diseases are at the forefront of technological innovation in healthcare.

“The use of big data and analytics, in particular, have significant potential to help speed diagnosis, a challenging area for the rare disease space,” Dr. Mayer says. “Delays in diagnosis are commonly experienced by rare disease patients and can lead to serious consequences for their health as well as to the wider healthcare system. In fact, many patients endure a multi-year journey before receiving an accurate diagnosis and beginning treatment.”

Christopher Anzalone, Ph.D., president and CEO of Arrowhead Pharmaceuticals, counts RNA interference (RNAi) as a powerful technology for the treatment of rare genetic diseases in 2018 and beyond.

“Advancements made over the last several years have set the stage for RNAi to move from an important scientific discovery

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PIERRE LAURIN
Prometic Life Sciences



to a groundbreaking therapeutic modality that improves the lives of patients,” he says. “At Arrowhead, we are developing RNAi therapy for the treatment of alpha-1 antitrypsin (AAT) deficiency, which is a rare, genetically inherited disorder associated with clinical liver and lung disease in both children and adults.”

Arrowhead’s ARO-AAT candidate uses the company’s proprietary Targeted RNAi Molecule, or TRiM, technology, which is designed to knock down the hepatic production of the mutant protein, termed Z-AAT, which is the cause of progressive liver disease in patients.

“Reducing production of the inflammatory Z-AAT protein is expected to halt the progression of liver disease and potentially allow it to regenerate and repair.”

Dr. Anzalone says the company will file a clinical trial application, or CTA, for its ARO-AAT candidate in first-quarter 2018.

“With ARO-AAT being just one example, we believe RNAi is on the brink of being a major disruptive treatment for rare genetic diseases in 2018,” he adds.

Lori Bartolomeo, executive VP, creative director, Dudnyk, agrees there is a desire to make a real difference in the lives of these patients.

“Rare disease patients and communities need as much advocacy and attention as they can get,” she says. “Not only do they need access to life-saving treatments, but also to specialists, services, and community support.



Innovation should not confine itself to rare or chronic disease states; rather drug development should be guided by scientific discovery and the question ‘what more is possible?’

ROBERT BAZEMORE
Epizyme

A Focus on Rare Disease

In a year-long series, PharmaVOICE has addressed a variety of market trends within the rare disease ecosystem. If you have missed any of these articles, please be sure to visit our online archive at <http://www.pharmavoice.com/archives/>.

January 2017	R&D
February 2017	Rare Disease Day
March 2017	Marketing
April 2017	Collaboration
May 2017	Patient Recruitment
June 2017	Supply Chain
September 2017	Patient Communities
October 2017	Regulatory Challenges

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We believe RNA interference will be a powerful technology for the treatment of rare genetic diseases in 2018 and beyond.

DR. CHRISTOPHER ANZALONE
Arrowhead Pharmaceuticals

They need partners that understand what they are going through, and who are willing to work with them to overcome barriers to diagnosis, access to treatment, and extension of survival. Our responsibility to patients is heightened in the rare disease space, and where there is greater need, there is greater reward.”

“The rare disease space represents an opportunity for companies to find that risk/reward profile that will allow it to serve its purpose, prosper and grow,” says Vincent Milano, CEO of Idera Pharmaceuticals.

Michael Thomas, principal at ZS, says patients also play a role in this process as they continue to seek early engagement with pharma and other stakeholders to establish funding and create research platforms for diseases they, or their family members, are facing.

Cost Under the Spotlight

While much continues to be done to encourage research into treatments for rare diseases, the cost of these specialty products has been under the spotlight.

Curable Co-Founder and CEO Lisa Boyette, M.D., Ph.D., says she would like to see companies that charge a lot for rare disease therapies introduce programs within their own organizations to keep pushing the envelope.

“If the cost of rare disease treatments is going to be high, I would like to see those dollars positioned to conclusively cure some of these diseases or prevent them, rather than have those dollars justified as covering the cost of development,” she says.

Dr. Cohen says it’s important to deliver a tangible, discernible, and measurable value to



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ANGI ROBINSON
Premier Research

both payers and insurance companies.

“Rare diseases often are associated with very high cost-of-care with patients requiring a large medical support team, frequent hospitalization, as well as very significant loss of productivity,” he says. “A successful new therapy must demonstrate that it ultimately reduces these costs while also improving the quality of life and longevity of the patient.”

However, according to Mr. Milano, important facts and context are omitted from the discussion about cost, including value to society and particularly people afflicted with rare diseases. He says with rare diseases there are few patients with an overall low-cost burden to the insurance company, which translates to low cost per member per month.

“Ultimately, if the headlines and politics win out over innovation, the only certain outcome is no one will endeavor to discover or develop the next solution for a rare disease,” Mr. Milano says.

To determine value, Mr. Blum says outcomes-based contracting will become the norm resulting in increased longitudinal tracking of patients across healthcare systems.

Pharma and biotech companies are engaging in much earlier planning and data collection for HEOR (Health Economics and Outcomes Research) information, Ms. Robinson says. This information is used to fully understand value and potential in real-world clinical practice and to support pricing and reimbursement decisions between drug makers and payers.

Stakeholders across the board are striving to rein in costs. For example, regulatory agencies are advocating for and developing

processes to support development strategies that may ultimately lower clinical costs for orphan drugs, such as seamless trial design, other complex adaptive designs, and novel endpoints, Ms. Robinson says.

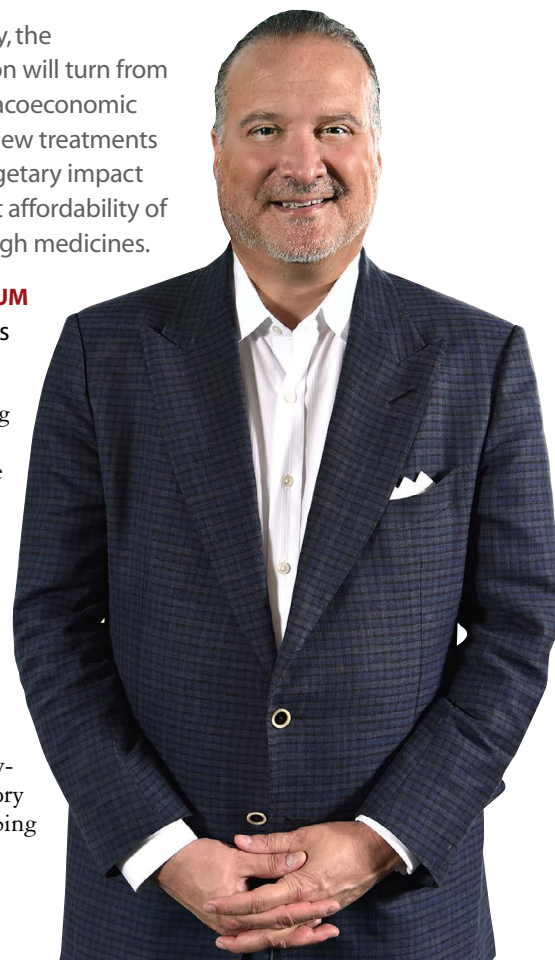
“Also, we’ve seen some increase in models such as pay-for-performance in rare disease drugs as well as those for more common conditions,” she adds.

Companies recognize the cost factor and most, if not all, companies with rare disease brands, create a variety of patient services focused on helping patients achieve available reimbursement and limit out-of-pocket costs, Mr. Thomas says.

He adds that where there is only one choice

Increasingly, the conversation will turn from the pharmacoeconomic impact of new treatments to the budgetary impact and patient affordability of breakthrough medicines.

ROBERT BLUM
Cytokinetics



of therapy, companies must help patients overcome both the fear of financial challenges as well as the challenge of the disease itself.

“This includes helping shepherd patients through the authorization process with their respective payer — commercial, Medicare, etc., aligning with other sources of financial offset, and a whole host of other services that provide benefit to the patient and their family while facing such a challenging medical condition,” Mr. Thomas says.

With an estimated 30 million Americans affected by a rare disease and approved or available treatments only available for a small percentage, the need for safe, effective, and accessible treatments is most important, Ms. Bartolomeo says.

“We as an industry need to rapidly roll out options for rare disease patients, because many of them don’t just need better options, they need any disease-modifying options,” Dr. Boyette says.

Dr. Mayer says for his company Shire, it is about bringing forward value.

“Rare diseases, our main area of focus, are often severe and complex conditions that are associated with lifelong suffering and a shorter lifespan,” he says. “Millions of people suffer from rare diseases, and many are children. Together they are one of the largest underserved patient populations in the world. Our medicines address rare diseases for which there are often no existing effective treatments, delivering significant value to patients, caregivers, healthcare providers, and society. Value can come in the form of a patient being able to work who could not do so before; a patient caring for themselves or their family whose disease rendered them unable to do so before treatment; a patient who now attends school, furthering their education, who could not do

so before. We are committed to responsibly pricing our products to reflect their value, and supporting patient access.”

Reducing costs, R&D resources, and time investment make the rare disease space attractive for drug developers, Mr. Laurin says, and that it also spurs innovation.

“Suddenly, that one product being developed using a new technology or approach based on new science coming out of great universities is viewed by someone who sees a potential application in another disease area.

“This has impact across the entire life-sciences industry,” he says. “We witnessed this impact in our own company, Prometic. When we first started to pursue the plasminogen congenital deficiency with Ryplazim, the clinical indication was pretty much limited to the unmet need in PLGD patients deficient from birth in plasminogen. But after a deep and thorough internal investigation on all the ramifications related to that specific protein and speaking to KOLs in this field, we realized the potential to investigate Ryplazim for other indications in which the body becomes acutely deficient in plasminogen, such as acute lung injury, and are now investigating it for several other indications.”

This is just one example of how creating the right R&D and regulatory environment can provide the right incentives and help create multiple opportunities within existing programs to address rare conditions that would not otherwise be advanced by companies, Mr. Laurin says.



With increased government incentives and a track record of increased clinical success and commercialization strategies, rare diseases are now viewed as promising indications for drug developers.

CARTER KELLER
GigaGen

“As rare disease incentive programs drive innovation throughout the entire biomedical industry, this will undoubtedly lead to further advancements in treatments for chronic and more prevalent diseases,” he adds.

Dr. Levin believes patients who have the opportunity to benefit from the wealth of innovation happening in the biotech and pharma industries should never have to suffer from lack of access to potential treatments due to cost.

“Often forgotten, a central point to the drug pricing debate is the need to address aligning pricing with value creation and innovation,” he adds. “This requires active dialogue between companies, government, physicians, and patients. We take this dialogue very seriously and have, even in our early days, looked at patient access. In addition, and beyond this value/pricing relationship, we should be working as an industry to strike a balance where we ensure executive compensation is more closely linked to the successful creation of breakthrough medicines and to real advances in healthcare. We need to create compensation tools to reward successful pipeline investment, not just top-line growth and short-term stock appreciation.”

Disruptive Innovations

Going forward, therefore, there will be



We are going to see many more drugs with novel mechanisms of action that address specific biological processes in these rare diseases.

DR. YUVAL COHEN
Corbus Pharmaceuticals

PIPELINE OF SELECT ORPHAN PROJECTS

Cancer	401	<div style="width: 100%;"></div>
Immunology	60	<div style="width: 15%;"></div>
Neurology	58	<div style="width: 14.5%;"></div>
Infections	32	<div style="width: 8%;"></div>
Musculoskeletal	26	<div style="width: 6.5%;"></div>
Cardiovascular	25	<div style="width: 6.25%;"></div>

NOTE: Figures represent Phase I,II,III and filed and approved

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increased focus on technologies that lead to disruptive treatments in rare disease.

“The pace of innovation is exceptional in many rare disease areas,” Jeremy Levin, D.Phil., CEO and chairman Ovid Therapeutics. “In rare disorders of the brain, this era is much like the revolution we saw in oncology that led to the explosion of immuno-oncology therapies in the 2005 to 2009 era of drug development. These technologies include imaging technologies, new genetic models, expression technologies, genetic manipulation, and novel cell models. Collectively, these technologies set the stage for totally disruptive treatments for the area of rare and orphan diseases of the brain. In addition, while recognizing that 80% of rare diseases have an identified genetic cause, the substantial technological advances taking place with next-generation gene sequencing, genomic technologies, and particularly CRISPR-Cas9 and modern AAV vectors are going to lead to new diagnostics and genetic treatments for patients.”

Dr. Levin believes that for all of these technologies to be effective, having deep knowledge of the underlying causes of rare diseases will be a critical component in drug development for these conditions. This, in turn, will allow researchers to identify types of therapies that will be most impactful. “The explosion of personalized cancer therapies is a great example of this success,” he notes. “Once we were able to understand the genetic underpinnings of specific

cancers, we were able to develop more targeted, more impactful therapies. I hope we are able to do the same with rare diseases.”

Orphan and pediatric diseases represent some of the most severe conditions with high unmet medical needs and relatively few, if any, treatment options. Mr. Laurin says this is certainly the case for plasminogen deficiency (PLGD), an ultra-rare disease that leads to hundreds of surgeries throughout an affected person’s lifetime and is the focus of his company’s lead program Ryplazim.

“To see that we could really do something meaningful with our proprietary technological platform to give these patients with no other options a chance at a normal life, as in the case of plasminogen deficiency PLGD, is why we are in this business — to use our technology to advance medicine.”

As technology is rapidly advancing, the industry will be able recognize more genetically defined diseases, says Robert Bazemore, president and CEO of Epizyme.

“For example, several cancer sub-types are being characterized as rare diseases,” he says.

“Smart companies will focus on a clinical development plan directed toward specific patient populations with a particular genetically defined disease. This is a significant component of the broader trend toward personalized therapeutics and creates the foundation for applying rational drug design tools to develop innovative therapies, and potentially using a companion diagnostic to select the right patients for treatment.”

Ms. Bartolomeo says recent advancements in whole genome sequencing, including exome sequencing, will give more patients and their families the opportunity to receive accurate diagnoses of their conditions than ever before.

“This technology can provide families long-awaited answers, and can give biopharmaceutical researchers a scientific understanding of specific genetic mutations that can lead to ground-breaking, life-altering therapies,” she says.

The future lies in big data technologies, Dr. Boyette believes, adding that with rare diseases it’s hard to pin down etiology using older approaches.

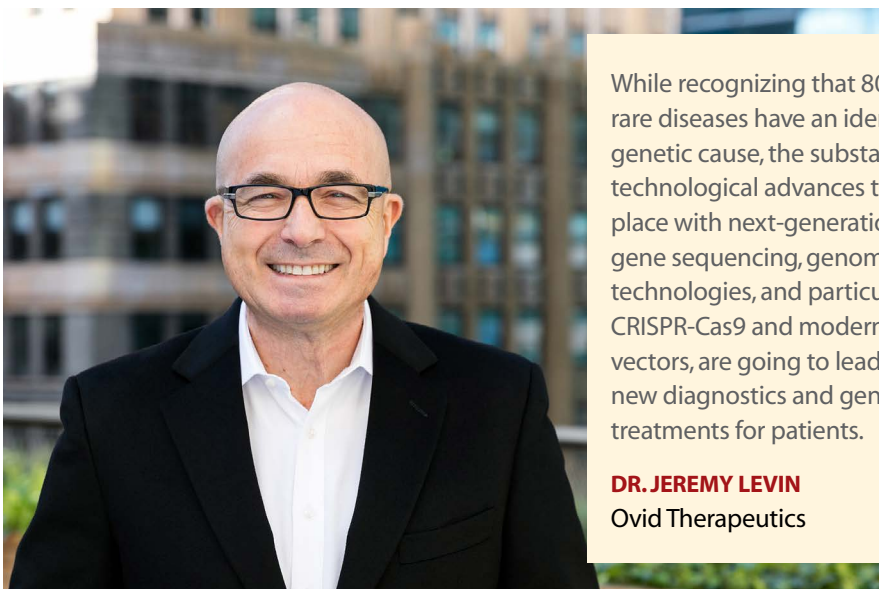
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ZS



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DR. HOWARD MAYER
Shire



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DR. JEREMY LEVIN
Ovid Therapeutics

“Next-generation sequencing enables discovery of disease-relevant mutations in rare diseases of purely genetic origin,” she says. “Multi-omics tool applications combined with systems biology analysis approaches are what is going to enable target identification in multifactorial rare diseases.”

“Data sharing technologies also enable us to reach, study, and work with rare diseases patients spread out across the country and around the world in ways we could not previously, incorporating health information and outcomes data from multiple EMRs into phenotypic catalogs that enable interpretation of all of the mechanistic data about the underlying biology of disease,” Dr. Boyette adds.

Dr. Mayer agrees that the industry is beginning to identify patients with rare diseases through the use of large data sets.

“For example, if a patient has a set of five symptoms, a doctor can run that combination of symptoms in his or her hospital’s system,” he says. “If the physician finds that four other patients in the hospital had the same combination of symptoms, they may know at that point to test for a rare disease. These technologies have the potential to dramatically improve the way we identify and diagnose underserved patients with rare conditions.”

According to Mr. Keller, three innovations

will be key to expanding the focus on rare disease drug development: more efficient drug discovery, greater fidelity of the genomic etiology of disease, and innovative regulatory paths for these drugs.

GigaGen has been focused on creating a platform that makes antibody discovery more efficient, shortening antibody discovery projects from a year to around six weeks by using the latest advances in microfluidic and genomic technologies.

“This type of drug discovery platform gets rid of the overhead and project failure most companies experience, enabling more discovery programs to be financed,” he says. “It has allowed us to quickly create a large pipeline of potential therapeutics against oncology targets at a cost that allows for taking risks in a rare disease space.”

Mr. Bazemore says as the collective understanding of genomics and epigenetics increases, there will likely be a great opportunity for innovation within genetically defined cancers.

“At Epizyme, we are focusing a significant amount of research toward sarcomas, a large group of very difficult-to-treat tumors — often characterized as a single class — when in reality sarcomas are a group of very different types of tumors,” he says. “We think there is



Our responsibility to patients is heightened in the rare disease space, and where there is greater need, there is greater reward.

Laurie Bartolomeo Dudnyk

an opportunity for innovation by using a genetically driven approach to develop targeted

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therapies for individual types of sarcoma based on the underlying genetic breakdowns, such as tumors characterized by a loss of the INI1 protein, which is implicated in the development of epithelioid sarcoma.

“Our hope for people living with genetically defined cancers is that new options will become available that provide improved efficacy because of the increased understanding of the underlying disease,” he continues.

Tools that allow for a greater understanding of the genetic causes of disease have been accelerating the discovery of drugs for specific patient populations, Mr. Keller says.

Dr. Cohen says there are likely to be many more drugs with novel mechanisms of action that address specific biological processes in these rare diseases.

Ms. Robinson points to work being done in cell and gene therapies and says technologies supporting regenerative medicine and the potential to address the underlying causes of different diseases will be transformative.

“The FDA recently gave recognition and support for this type of product potential by establishing the Regenerative Medicine Advanced Therapy designation, adding to the existing expedited programs,” she says.

Mr. Blum says gene-directed treatments may be disruptive but not always curative thereby opening the door for chronic care treatments for certain rare diseases once thought to be fatal.

“More companies are pursuing discovery/development of novel mechanism treatments for rare diseases given the explosion of genomic data, which is being harnessed for gene-directed treatment strategies,” he says. “As a result, we can expect more precision medicine strategies to shift the competitive dynamic away from mechanism to modality — cell therapy vs. gene therapy. Complementary treatments that extend survival, preserve quality of life, reduce functional decline, and treat symptoms will prove valuable.”

Dr. Mayer says one of the biggest advancements in medicine in general is personalized medicine, especially the ability to diagnose patients and personalize treatments through gene sequencing.

With gene sequencing, treatments can be tailored to each patient based on his or her DNA.

“Personalizing treatments is important for rare diseases since many are genetic in nature,” he says. “This personalization is already starting to cross over to therapies for chronic diseases, which are the main cost drivers of healthcare in the United States and globally. Increased use of gene sequencing will further our understanding of the genetic variants involved in chronic disease, and it’s exciting to

FAST FACT

ORPHAN DRUGS WILL ADD \$95 BILLION IN ADDITIONAL SALES BY 2022.

Source: Evaluate

imagine how this will improve the quality of life for so many patients who are in dire need of effective treatment.”

Mr. Milano adds that while this type of disruption in treatment alternatives will confront many challenges around the economics and fundamental ways in which biotech and pharmaceutical companies are measured, the impact for patients is by far the most important factor and should be the driving force behind this pursuit.

The Rare-Common Connection

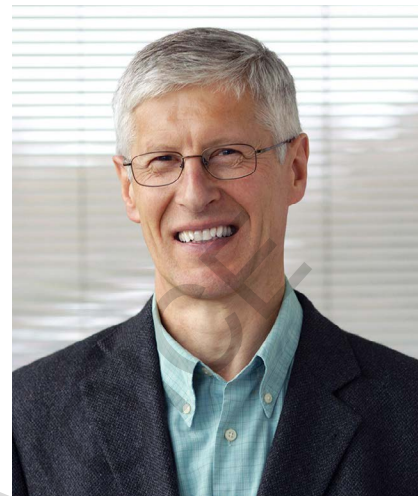
Scientifically, the processes in exploring and understanding rare diseases have strong correlation with more common conditions, and the research contributes to understanding of body processes and pathways relevant to greater understanding of the pathology of common diseases.

Deep study of rare genetic diseases has led to a better understanding of the evolutionary process for more common but complex chronic conditions that started as severe and more simplified diseases, and it influences industry’s approach to how these chronic diseases should be treated, Ms. Robinson says.

“An example of this is the study of familial hypercholesterolemia, which made the genetic connection between heart attacks and cholesterol and contributed to the development of statins,” she says. “The attention focused on specific rare diseases is creating greater disease awareness, which in turn may result in more accurate and timely diagnosis. In some cases, this awareness, combined with better means of diagnosing patients and greater access to modernized electronic medical records in areas previously uncharted, demonstrates a greater global prevalence than was originally understood — for example, with celiac disease.”

Dr. Boyette concurs, noting that rare diseases can often serve as gateways to new drug development because of the regulatory advantages put in place around orphan conditions with those therapies proving useful for larger populations with phenotypically similar chronic diseases.

“For example, development of antifibrotics



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DR. IGOR GONDA
Aradigm Corp.

for rare liver and biliary disease may yield therapies that are useful in all kinds of chronic liver conditions that lead to cirrhosis,” she notes. “More than 1 million people a year die of cirrhosis worldwide, so that’s potentially a very big deal even though some of these programs start with a focus on a rare disease as a proof of concept.”

Dr. Levin says because many rare diseases impact people at birth or at very young ages, it was important for his company to establish drug development programs targeted at younger patient populations and treatments that will work throughout the life of the patient, which also helps them think about how they develop drugs for chronic conditions.

“At Ovid, we strive to satisfy all the medical needs of the patient rather than just focusing on one symptom; this is how we will truly make a difference in the life of a child and for the families who are living with a rare disease,” he says. “This same mindset is what is needed to take a holistic approach to treating chronic diseases, rather than focusing on one symptom, we must look at how treatments fit together to benefit the overall health of the patient.” ^{PV}

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