FDA Approves 22 Novel Drugs IN 2016

In 2016, novel drug approvals included the first treatment for patients with spinal muscular atrophy, Spinraza; the first drug approved to treat Duchenne muscular dystrophy, Exondys 51; Nuplazid, a new drug to treat hallucinations and delusions in some people with Parkinson's disease, Ocaliva, a new drug to treat patients with a rare chronic liver disease known as primary biliary cirrhosis; and Epclusa and Zepatier, two new treatments for patients with hepatitis C.

The field also includes new treatments for patients with ovarian cancer, bladder cancer, soft tissue sarcoma, and chronic lymphocytic leukemia—as well as two new diagnostic agents for detecting certain forms of cancer.

THE GLOBAL BIOTECHNOLOGY SERVICES OUTSOURCING MARKET IS EXPECTED TO REACH $92.9 BILLION BY 2025, A CAGR OF 8.7%.

Grand View Research

R&D Returns Continue TO DECLINE

The pharmaceutical industry continues to face regulatory and reimbursement hurdles weighing on the research and development returns of pharmaceutical firms this year, finds the seventh annual pharmaceutical innovation study by the Deloitte UK Centre for Health Solutions.

Key findings:

- Annual projected pharma R&D returns continue to decline to 3.7%
- Peak sales per asset fell 11.4% year-on-year since 2010
- Costs to bring a product to market stabilized in 2016, from $1,576 million in 2015 to $1,539 million
- Smaller pharma companies have seen a decline in overall performance, but on average they continue to outperform their larger counterparts, generating returns up to three times higher

TRENDS IN BIOPHARMA INNOVATION

The surge in biopharmaceutical innovation over the past few years is expected to continue based on the number of quality molecules in late-stage clinical development.

Over the last 20 years, a total of 667 innovative biopharmaceuticals have launched in the United States, finds a new report from QuintilesIMS. The characteristics of biopharmaceutical innovation have evolved in some respects over the past two decades. Most notably, drugs for the treatment of patients with cancer have increased their share of all launches from 11% in the 1996 to 2000 period to 28% in the most recent five-year period. During the same period, drugs launched with an orphan indication rose from 21% to 42% of all launches. The proportion of new biopharmaceuticals that are biologic in nature and primarily prescribed by a specialist also rose during the past 20 years.

Other findings:

- Nearly three-quarters of the molecules were launched by a company different from the one that filed the original patent.
- The average time from original patent filing until U.S. launch for all molecules is 12.8 years, with upper and lower quartiles of 16.3 and 8.2 years, respectively.
- The majority of drug launches achieve very modest levels of average annual sales in their first five years on the market. Over the 20-year period, 62% of the launches averaged less than $100 million in average annual sales during their initial five years following launch.

Novartis Launches SMS FOR LIFE 2.0 IN NIGERIA

Novartis has launched of an innovative technology-based healthcare program called SMS for Life 2.0 in Kaduna State, Nigeria. The program aims to increase the availability of essential medicines and improve care for patients across the region by using simple, available, and affordable technology. SMS for Life 2.0 is a joint public-private partnership led by Novartis and supported by its partners, the Kaduna State Ministry of Health, and Vodacom. The program uses smartphones and tablet computers to improve access to medicines and increase disease surveillance, helping to provide better care for patients.

SPEAK Out

Provided by: Worldwide Clinical Trials

Maximizing Patient Recruitment in RARE DISEASE RESEARCH

Jeffry Zucker,
Vice President,
Feasibility and
Recruitment
Optimization,
Worldwide Clinical
Trials

Barbara Zupancic,
Director, Global Patient
Recruitment and
Retention,
Worldwide Clinical Trials

Creating a pharmaceutical development program for the treatment of a rare disease is challenging. From recruiting and retaining patients and investigators, through to regulatory uncertainties and limited understanding of the natural history of the proposed indication due to few observational trials studying disease progression, there are many considerations and hurdles to overcome.

Despite permissive science, innovative trial methodology, evolving regulatory sentiments and increasingly sophisticated commercialization all supporting rare disease drug development in recent years, patient recruitment can become one of the most daunting challenges in rare disease drug development.

Maximizing Participation

Sourcing and identifying participants for rare disease studies can be an obstacle for researchers due to smaller and geographically dispersed patient groups.

Small patient numbers mean that the involvement of every single patient is vitally important. It also means the impact that clinical endpoints from a limited number of patients may have on program development is exceptional. For this reason, engaging sites, investigators, and patients to confirm acceptance of the study design is vital. Proactively engaging all stakeholders can foster a collaborative approach that not only facilitates recruitment, but also retention and commercial value long-term.

Advocacy Outreach

Advocacy groups exist for most rare disease indications. Contract research organizations (CROs), sponsors and investigators should engage with these groups, especially during the initial stages of a protocol design, because having their cooperation can be crucial to the success of a trial. These groups usually include patient caregivers or relatives, as well as physicians and key opinion leaders (KOLs) in the specific indication. By taking the time to meet and speak with members of relevant groups, investigators can gain valuable insight on a particular condition, including what life is like for patients, as well as what is important to caregivers and others in the patient's network

Due to the rarity of some conditions, there may be no established advocacy or patient groups. In this case, general registries such as the Global Rare Disease Patient Registry and Data Repository, entities such as the National Organization for Rare Disorders and the European Organisation for Rare Diseases, as well as resources such as Orphanet, are invaluable as a first step in site identification and selection. Researchers can also make use of international rare disease research consortiums, such as Eurodisk and the National Center for Advancing Translational Sciences, which work to foster international collaboration through preclinical and clinical research in rare diseases.

Supporting Recruitment

In order to further support recruitment, it is also crucial for researchers to gain the trust and buy-in of patients and their caregivers. More so than with non-orphan conditions, sufferers of rare diseases, as well as their caregivers, are typically well informed about their condition and the latest research. Ultimately, if they do not buy-in to what the team is trying to achieve with the study, or the suggested approach, then there is little hope of recruiting the patient.

Researchers should bear in mind that physicians treating sufferers of rare diseases often have a strong relationship with their patient, which can sometimes result in a reluctance to enroll them in clinical trials at sites outside of their jurisdiction. For this reason, a physician who is fully engaged and informed about a study from the offset is more likely to share motivations and recommend to patients that they participate in the trial. Additionally, physicians and investigators specializing in rare disease are typically well connected on a global scale. Researchers should proactively reach out to the most influential in the relevant indication in order for their study to optimize patient accrual.

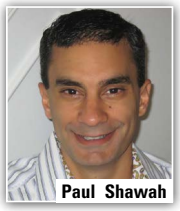
As with any trial, the whole research team must be fully engaged from the onset of the study and throughout in order to maximize patient recruitment. By involving the team in discussions and planning as soon as possible, sponsors can ensure everyone feels engaged and that they are adding value to the protocol, and as a result, are more likely to pass this enthusiasm and understanding along to potential participants.

Conclusion

While it is clear that creating a drug development program for the treatment of a rare disease is no easy task, there are a number of steps researchers can take, particularly when it comes to patient recruitment. By putting the patient and their care network at the heart of the study through careful integration of design and operations, and by engaging with influential KOLs, regulators, patients and their care network from the offset of the study, researchers can improve drug development efforts in this field.

For further information on strategies to recruit and retain rare disease patients, visit www.worldwide.com.

Top Global Pharma Companies Establish NEW INDUSTRY STANDARDS GROUP


Paul Shawah

Veeva Systems and six of the top 25 largest global pharmaceutical companies have announced the formation of Align Biopharma, a group dedicated to setting technology standards that will make it faster and easier for healthcare professionals (HCPs) to connect with the life-sciences industry. Founding members, with input from across the industry, will develop open standards and solutions for companies to streamline how HCPs get the drug and treatment information they need to deliver improved care to patients.

"Common industry standards can make it more effective and efficient for life-sciences companies and healthcare professionals to connect," said Patrick Retif, VP IT, Global Commercial at Allergan. "Working together can help harmonize digital engagement and information access across the industry and create a better experience for our shared customers."

The rise of specialty drugs to treat complex diseases is creating a greater need for HCPs to have more timely and tailored information. As the number of new drugs increases so does the amount of information healthcare professionals require to facilitate patient engagement. There is significant potential for biopharmaceutical companies to use digital technology to inform HCPs on new or more complex treatments.

Initially, Align Biopharma will focus on developing two new standards to facilitate seamless digital engagement and simplify the HCP experience:

Identity management — definition of an identification and authentication standard to enable a single sign-on for HCPs to access online content — including websites, portals, virtual events, or webinars — across all companies.

Consent and communication preferences — definition of standards for consent and preference management so that there is consistency in how HCPs specify communication preferences with each company.

"Digital is transforming how life-sciences companies and HCPs collaborate and interact," says Paul Shawah, VP of commercial cloud strategy at Veeva. "With the proliferation of advanced treatments and digital channels, adhering to industry standards will simplify the challenges doctors face in getting the right information quickly."

Biopharmaceutical M&A EXPECTED TO SOAR

The biopharmaceutical industry's desire for inorganic growth is expected to intensify an already heated M&A environment in 2017. This is according to the EY M&A Outlook and Firepower Report 2017. Key findings:

- Total M&A volume across the biopharma industry exceeded $200 billion in 2016, a level unheard of prior to 2014 but in line with the deal volume of the previous two years. Big pharma was responsible for the lion's share of this deal activity with over 70%.
- Falling equity valuations and debt raised to fuel previous years' M&A have resulted in roughly a 20% decline in firepower across the industry. Specialty pharma and big biotech companies have experienced the largest declines, down 62% and 24%, respectively, while big pharma dropped only 17%.

PHARMAVOICE.COM

WEBINARS

» **Digital by Design – 3 Ways to Retool Your Business with Technology in 2017**
Sponsored by: DocuSign

WHITE PAPERS

» **Making Digital Health Personal — Best Practice Digital Tools to Deliver Value-Based Care**
Provided by: Atlantis Healthcare

MORE WHITE PAPERS

» **Mental Health: An Advocacy Perspective on Sustained Partnerships**
Provided by: inVentiv Health

» **Mental Health: A Payer Perspective on Advocacy Concerns**
Provided by: inVentiv Health

New Pharma Line Up

CHRISTI SHAW TO REJOIN LILLY


Christi Shaw

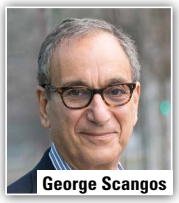
Former Novartis President Christi Shaw is returning to Eli Lilly and Company, where she started her career in 1989, to lead the Bio-Medicines business beginning April 3. Ms. Shaw will succeed David Ricks, who became Lilly's president and CEO on January 1. In her new role, Ms. Shaw will lead important areas of potential growth for Lilly: immunology, neurodegeneration, and pain, as well as many of the company's established brand products.

EMMA WALMSLEY WILL TAKE HELM OF GSK

At GlaxoSmithKline, Emma Walmsley takes over the reigns from Andrew Witty as CEO in March. Ms. Walmsley has served as CEO of GSK Consumer Healthcare since 2011.

Ms. Walmsley joined GSK in 2010 from L'Oreal where, over the course of her 17-year career, she held a variety of marketing and general management roles in the UK, Europe and USA. From 2007 she was based in Shanghai as General Manager, Consumer Products for L'Oreal China.

FORMER BIOGEN CEO GEORGE SCANGOS HEADS BIOTECH START UP


George Scangos

Vir Biotechnology launched in January to develop cures, treatments, and preventions for challenging infectious diseases. Former Biogen CEO George Scangos is heading a team of scientific and industry leaders. The company is headquartered in San Francisco.

Vir seeks to take a new approach, using breakthroughs in immune programming to manipulate pathogen-host interactions and taking a multi-program, multi-platform approach. Lead investors include ARCH Venture Partners and the Bill & Melinda Gates Foundation.

PharmaVOICE 100

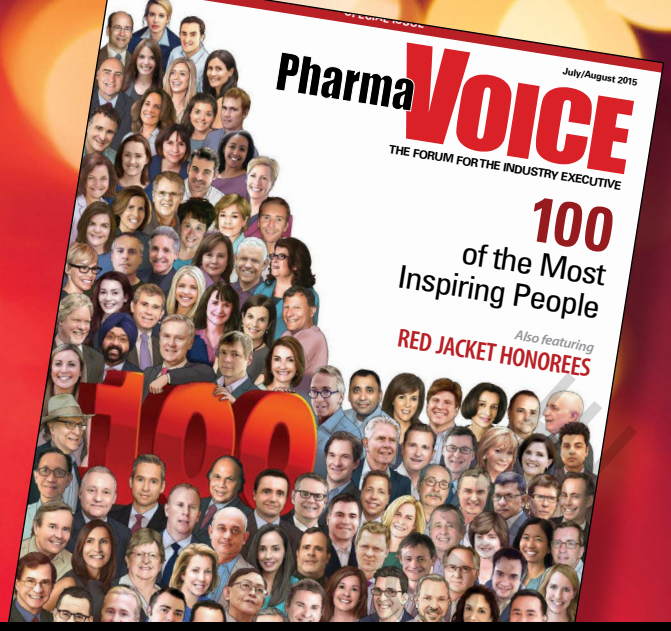
SAVE THE DATE

PharmaVOICE 100 Celebration

PharmaVOICE is excited to announce the third annual **PharmaVOICE 100 Celebration** – an event that convenes the most influential and inspirational leaders from across all sectors of the life-sciences industry. PharmaVOICE's positioning allows us to bring together these inspirational leaders in a unique format allowing for much-needed collaboration through sharing ideas, insights, and business perspectives, while enjoying the opportunity to expand personal and professional networks.

Thursday, September 14th

Apella Event Space
Riverpark, a Tom Colicchio Restaurant
The Alexandria Center for Life Science
450 East 29th Street
New York, NY 10016



WHO INSPIRES YOU? WE WANT TO KNOW!

The PharmaVOICE 100 are leaders in research and development, marketing, technology, creativity, strategy, medicine, etc. They positively impact their peers, their colleagues, their companies, their communities, as well as the industry at large through their actions.

The PharmaVOICE 100 set industry trends, their passion and conviction create opportunities out of obstacles. They are innovative and have the ability to think outside-the-box. They develop breakthrough strategies, products, and services. They are known for pioneering new paths and lifting their companies to new heights. They also take the time to mentor the next generation of industry leaders.

NOMINATE SOMEONE WHO INSPIRES YOU!

Deadline: April 1st

www.pharmavoice.com/100