Addressing the Needs of THOSE WITH RARE DISEASES

Peter Saltonstall, President and CEO of the National Organization for Rare Disorders (NORD), talks about recent orphan drug approvals.

PV: 2014 was a record year for orphan drug approvals. What are your predictions for the orphan drug space for 2015?

Saltonstall: The FDA's Center for Drug Evaluation and Research (CDER) approved 41 novel new drugs in 2014 of which nearly 40% are orphan drugs for treating patients with rare diseases. This is the highest yearly approval since the Orphan Drug Act was passed in 1983. I'm proud of the work we are doing and that one of the 41 new drugs resulted from a NORD research grant program. We have significant unmet medical needs in rare diseases, and there are many companies that are interested in trying to help patients. Additionally, the FDA has worked hard to continue to improve and approve these drugs as quickly as possible.

I believe we are going to see more gene therapies. There are a lot of companies working in this space, and the companies that NORD works with are doing some exciting things, especially in areas where there are unmet medical needs.

I'm hoping this strong approval trend will continue and that the agency receives the appropriate funding. The FDA has more than 650 openings right now, which is a 20% open rate. This gap can impact review divisions and how quickly they approve drugs. Appropriately funding the FDA and making sure that the agency can recruit the people needed in the review divisions is critically important.

PV: How is the industry changing its focus in terms of developing new drugs for rare diseases?

Saltonstall: Interacting with patients is something that companies are doing more of, especially in the orphan space. In many instances, patients within the orphan space almost know more about the drugs than clinicians do; they have researched the disease area since they were diagnosed or their family members were diagnosed. In many instances where there weren't any therapies, patients have worked with the NIH and industry to move research forward. The patient-company partnership continues to be extremely important. NORD is helping to match some of the smaller, patient organizations with companies and advocates for appropriate funding for the FDA.

There should be the same consideration to the regulatory pathway for follow-on versions of NBCDs as there are for biosimilars, including post-marketing surveillance studies.

PV: What do you see as some of the opportunities for developing therapies for rare diseases?

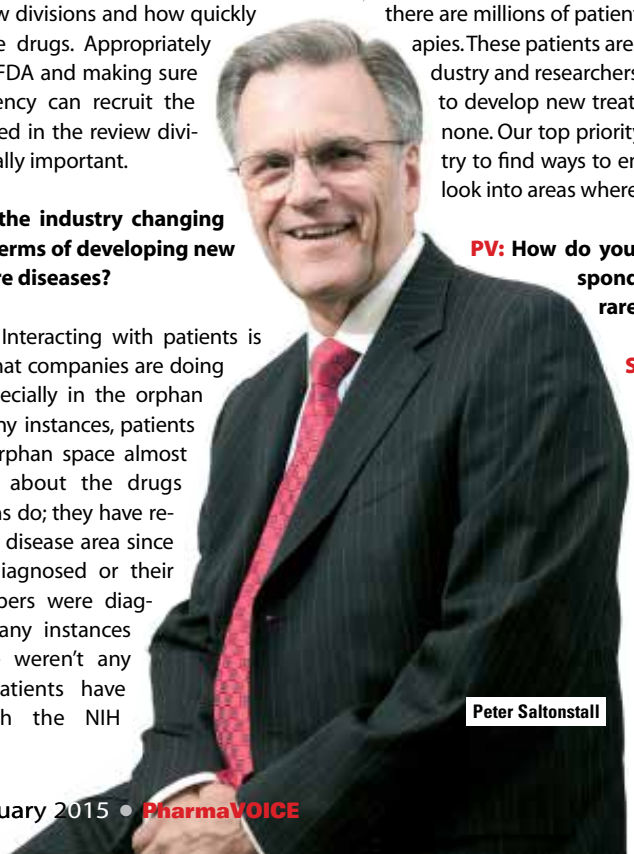
Saltonstall: Companies are working on repurposing existing drugs to help bring them to market more quickly. But incentives and the regulatory pathway for companies to be able to repurpose existing drugs need to be in place.

It's also important for companies to conduct research in rare diseases where there are no therapies. There are about 450 approved orphan products for about 350 different diseases. While another therapy for Gaucher's disease might be important, I'd like to see therapies where there are none right now. There are 7,000 rare diseases and there are millions of patients waiting for new therapies. These patients are willing to work with industry and researchers to get to the next step to develop new treatments where there are none. Our top priority is to work with industry to find ways to encourage companies to look into areas where there are no therapies.

PV: How do you think payers will respond to more therapies for rare diseases?

Saltonstall: NORD hosted a group of payers to have a conversation about reimbursement issues. Surprisingly, they said they did not have as much concern about orphan products and orphan product pricing as they did with specialty drugs. In the marketplace, orphan products often get lumped into the specialty market. Payers are looking closely at specialty drugs, which are expected to double in growth compared with orphan drug growth.

IMS released a report showing that orphan drugs only account for between 4.8% and 8.9% of overall drug spending. Looking toward 2019, orphan products will still only account for 8.5% or 8.7% of the overall marketplace. PV



Peter Saltonstall

NORD
National Organization for Rare Disorders

16 Orphan Drugs Approved in 2014

- **Beleodaq by Spectrum**
  Peripheral T-cell lymphoma
- **Blincyto by Amgen**
  Acute lymphoblastic leukemia
- **Cerdelga by Genzyme**
  Type 1 form of Gaucher disease
- **Cyramza by Lilly**
  Advanced stomach cancer
- **Esbriet by InterMune**
  Idiopathic pulmonary fibrosis
- **Hetlioz by Vanda**
  Non-24 hour sleep-wake disorder
- **Impavido by Paladin**
  Tropical disease Leishmaniasis
- **Keytruda by Merck**
  Advanced melanoma
- **Myalept by AstraZeneca**
  Complications of leptin deficiency
- **Northera by Lundbeck**
  Orthostatic dizziness
- **Ofev by Bristol-Myers Squibb**
  Idiopathic pulmonary fibrosis
- **Opdivo by Boehringer Ingelheim**
  Advanced melanoma
- **Sylvant by Janssen Biotech**
  Multicentric Castleman's
- **Vimizim by Biomarin**
  Enzyme replacement therapy for Morquio A
- **Zydelig by Gilead**
  Three types of blood cancers
- **Zykadia by Novartis**
  Late-stage non-small cell lung cancer

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John Van Johnston | Director, Business Development Product Access Support Services
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