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**Letters...**

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## Strong Together

In the rare disease community there is saying: Alone we are rare, together we are strong. Wendy White, senior VP, for rare diseases, at Dohmen Life Science Services, a board member of Global Genes, and the president of the Healthcare Businesswomen's Association (HBA), shared her personal rare disease story at the HBA's recent Woman of the Year event. She talked about how she became a detective mom when her youngest child was born with an undiagnosed rare condition called Nail-patella syndrome, a disease that is characterized by abnormalities of the nails, knees, elbows, and pelvis. In the case of her daughter, the condition was manifested in her knees, which doctors said would impact her ability to ever climb stairs. Now, 13, Casey is not only able to climb stairs, but she plays soccer and actively participates in many other pursuits. Ms. White, like many mothers and fathers, spent years searching for answers. Her pursuits led her to change her company's business model — Siren Interactive — to address the lack of knowledge around rare diseases. Siren was enveloped into Dohmen earlier this year.



Ms. White's journey is just one of the thousands of stories of inspiration within the rare disease community. To draw attention to rare diseases as an important public health issue that cannot be ignored, Rare Disease Day was established in the United States seven years ago to be held on the last Thursday in February. As part of this year's celebration the organization launched Handprints Across America photo gallery, which accepts photos and submissions for patients and caregivers to tell the world how rare diseases affect them.

In the past few years, rare diseases have generated a great deal of interest from pharmaceutical and biotechnology companies; in 2014, orphan drugs constituted 17 of the 41 new molecular entities approved by the FDA. An average of 140 drugs have been designated as orphan drugs by the FDA each year over the past decade, compared with 64 in the previous 10 years, according to the PhRMA. Additionally, President Obama announced that he would devote \$215 million of his 2016 budget to a new Precision Medicine Initiative, including funds for a database at the National Institutes of Health (NIH) that will house genetic and lifestyle information, biological samples, and links to EMRs voluntarily contributed by 1 million Americans.

As we were putting together this month's cover story — Rare Disease: A Flourishing Market — we caught up with Andrew Robbins, chief operating officer of Array BioPharma, who says increased investment into rare diseases is also building awareness, helping patients become more sophisticated about their own diseases and how they should be treated. Working directly with patient advocacy groups can allow for companies to enlist a smaller salesforce and bypass costly marketing expenses typical to primary care drugs. A sophisticated patient is also more aware and willing to come onto clinical trials for new therapies, reducing the cost and time of clinical trial recruitment, ultimately helping to advance the scientific knowledge around these diseases.

Taren Grom  
Editor

### Their word...

**DENISE MYSHKO**  
Managing Editor



*New efforts by Congress and pharma companies may one day mean that all patients with rare diseases will have a treatment option.*

**ROBIN ROBINSON**  
Senior Editor



*Patients are expecting the same level of customer service from their healthcare process as they do from an Amazon purchase, and pharma needs to be a part of that solution.*

**KIM RIBBINK**  
Features Editor



*Poland's size, geography, stability and bright outlook make it an exciting market for pharmaceutical companies.*

Coming in  
**JULY/AUGUST 2015**



The July/August issue features 100 of the most inspiring individuals in the life-sciences industry as determined by you, our readers. Your personal accounts that detail how these outstanding individuals throughout the life-sciences industry — executives, peers, colleagues, clients, etc. — motivate you and inspire change in all sectors of healthcare, make this special issue come to life. See who's on the list this year.



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