

DIA 2013 49th Annual Meeting "Advancing Therapeutic Innovation and Regulatory Science" June 23 – 27, 2013 Boston Convention and Exhibition Center Boston, MA

Session 360 – Rising to the Challenge of Developing Novel Orphan Medicines for the Global Market

This year marks the 30th anniversary of the Orphan Drug Act. Session 360, *Rising to the Challenge of Developing Novel Orphan Medicines for the Global Market*, provided a gamut of perspectives from research, regulation and patient groups.

Session chair Cecil Nick (PAREXEL Consulting) laid out a fundamental difficulty of orphan drug development, patients expect the drug to be safe and regulatory entities must decide if the benefit of the drug outweighs the risk with the evidence it is provided. But in the context of rare disease, amassing this evidence is an incredible challenge.

Dr. John Ziegler (Premier Research Group) provided a comprehensive introduction to challenges in developing orphan drugs for rare disease and highlighted NIH's response to these challenges. He also introduced and set the stage for Audrey Gordon, Esq. of the Progeria Research Foundation (PRF).

Ms. Gordon shared the inspirational story of PRF. In 1998 her nephew Sam was diagnosed with Progeria. Her sister, Dr. Leslie Gordon founded PRF in 1999, by 2003 the mutation in the LMNA gene to cause the disease was identified, in 2007 single arm open label clinical trials began and in 2012 they published a paper in PNAS reporting "every single child on the Lonafarnib improved with treatment." Ms. Gordon covered some of the ways PRF dealt with a small and geographically diverse patient population, clinical trial design and other challenges; she also highlighted crucial collaborators.

Dr. Spiros Vamvakas (European Medicines Agency) followed with a European regulatory perspective on the framework for orphan drug development. He highlighted the 10-year market exclusivity and additional two-year exclusivity for pediatric drugs as one of the many incentives EMA supports. Dr. Vamvakas also highlighted the initiatives in research, the European Horizon 2020 and International Rare Disease Research Consortium (IRDiRC).

Dr. Gayatri Rao (FDA) concluded the session with what the FDA is doing in regulation and research to promote the development of products for rare diseases. Notably, she pointed out that a major challenge is correct diagnosis of rare disease and the need for good diagnostic tests. Dr. Rao highlighted a new final rule of the Orphan Drug Act, "hot of the press" as of June 12th, 2013. On the research side, she highlighted one of the largest grants programs at the FDA, the Orphan Products Grants Program, which funds clinical development for both academic and industry efforts.

Being new to the research and regulation of rare disease, I was awestruck by the unique challenges faced by this community. Regulatory, clinical and research areas together with patient advocacy groups continue to rally and reevaluate their efforts to address the clear need for orphan products.

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