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Access Viewpoint

Rare diseases, orphan drugs, specialty medicines: success brings payer scrutiny

On Rare Disease Day 2016, patients and their organizations all over the world held activities on the last day of February, as they've done every year since 2008, to raise awareness of the over 6,000 rare diseases and their impact on the lives of patients.[1]

How well have they succeeded? Let's take a quick look at the current therapies for rare diseases, their growth and costs—and then consider how payers are reacting.

Rare diseases, when taken together, are not that rare at all

Before the Orphan Drug Act was passed in the US in 1983 to incentivize the development of drugs to treat rare diseases, only 38 orphan drugs were approved.[2] Among the new drugs approved by the FDA in 2015 alone, almost 50% (21 of 45) are orphan drugs.[3] Three orphan drugs are forecast to achieve blockbuster sales by 2020.[4] Worldwide orphan drug sales are predicted to grow to $178 billion and 20% of global prescription sales by 2020—twice as fast as the overall drug market, with a median cost per patient almost 14 times higher.[2]

If there ever was a time when the individual cost of these life-changing drugs was largely accepted by payers, that moment has passed. The aggregate growth of orphan drugs is bringing payer scrutiny to treatments that may previously have gone unexamined because they were directed toward small populations. As PhRMA itself has said, "Rare diseases, when taken together, are not that rare at all."[5]

Among the trends giving payers cause for concern:

- The FDA has granted orphan designation to over 100 drugs with existing approvals for more common diseases[6]
- About 15% of orphan drugs analyzed in one study had subsequent launches for additional rare diseases[7]
- Drugs that originally targeted a very specific subset of a population can subsequently be expanded beyond the initial indication
  - According to Mary Dorholt, clinical practice leader at Accredo, ESI's specialty pharmacy, almost 20 orphan drugs have enjoyed blockbuster sales on expanded US indications[8]

The orphan "druganaut"[9] requires a thoughtful and calibrated manufacturer response to burgeoning access challenges. It's important to payers that drug makers clearly identify the clinical unmet need of the disease, target only the relevant members who are at specific risk, and show transparency through shared planning for the approval of broader indications.

As specialty medicines of all kinds continue to crowd the marketplace, reimbursement becomes more complex (a problem for manufacturers, healthcare providers, and patients) and distribution more limited (making these drugs more expensive for payers).

Designed to facilitate reimbursement, education, and adherence, as well as provide seamless distribution, hubs are fast becoming a must for manufacturers launching specialty products. Minimal services include providing assistance for healthcare professionals and their office staff, supporting patients and caregivers, and facilitating communications with specialty pharmacies. But today, there's growing pressure on manufacturer hubs to deliver even more value. Fully integrated digital benefits investigations and prior authorization handling are at the top of many prescriber wish lists, along with simplified, HIPAA-compliant patient status tracking and a proven ability to reduce phone calls and treatment delays. Hubs are quickly moving toward providing a truly automated, single point of contact across the spectrum of use.

To learn more, please **contact Andrew Gottfried at agottfried@entreehealth.com or 212-896-8026.**

References: 1. Rare Disease Day®. http://www.rarediseaseday.org/article/what-is-rare-disease-day. Accessed March 1, 2016. **2.** Evaluate Pharma®. Orphan drug report 2015. http://info.evaluategroup.com/rs/607-YGS-364/images/EPOD15.pdf. Accessed March 4, 2016. **3.** Pagliarulo N. Looking ahead at orphan drugs' future: more breakthroughs, exploding sales. http://www.biopharmadive.com/news/looking-ahead-at-orphan-drugs-future-more-breakthroughs-exploding-sales/413974/. Accessed March 4, 2016. **4.** Thomson Reuters. Drugs to watch 2016. http://images.info.science.thomsonreuters.biz/Web/ThomsonReutersScience/%7B7e677448-1313-40ae-9978-6be1a8532a08%7D_A4_MarketInsight_Report_0213_012__edits_2.pdf. Accessed March 4, 2016. **5.** PhRMA. Rare diseases: a report on orphan drugs in the pipeline. 2013. **6.** US Food and Drug Administration, *A Valuable Resource for Drug Developers: The Rare Disease Repurposing Database (RDRD).* http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/ucm216147.htm. Accessed March 4, 2016. **7.** Reuters. *The Economic Power of Orphan Drugs*, 2012. http://thomsonreuters.com/business-unit/science/subsector/pdf/the-economic-power-of-orphan-drugs.pdf. Accessed March 4, 2016. **8.** Shelley S. The business of orphan drugs is booming. *Pharmaceutical Commerce.* August 26, 2015. **9.** Orphan Druganaut Blog. 10 new FDA orphan drug designations: week of 06/08/15. https://orphandruganaut.wordpress.com/. Accessed March 1, 2016.