Qral Group

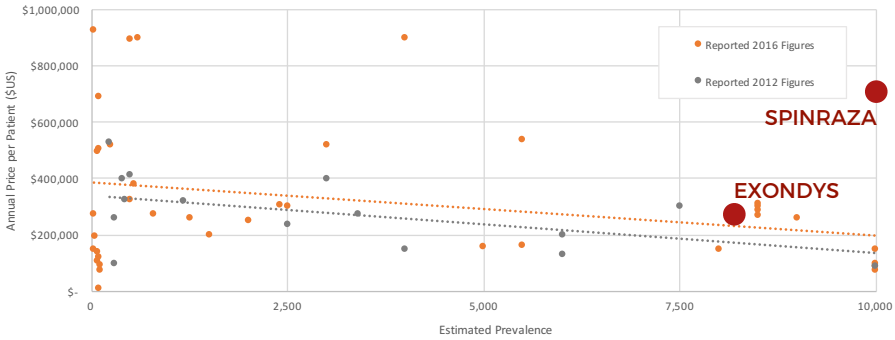
$200,000 the new max? Payers start to redefine "Proven and Medically Necessary"

Qral Group conducted a series of payer roundtables in Denver, shortly after the AMCP (Academy for Managed Care Pharmacy) Annual Meeting in March of 2017. In one of those roundtables, we asked Medical and Pharmacy Directors to share their perspectives on how the coverage and reimbursement environment for orphan/ultra-orphan products is evolving.

This year, many new themes emerged. Hints of these themes originally reported in our "Observations from 2017 JPM" (published 1/16/17). But now, Medical & Pharmacy Directors are more confident in putting forward new definitions for what constitutes Proven and Medically Necessary.

Evolution of the Orphan Product Landscape (2016 vs. 2012)

The relationship between annual therapy cost & price is commonly used in investor reports to evaluate price levels for ultra orphan brands.



- The AHIP reported 20 products priced >$200,000 per year
- Of those 20 products, 5 have launched since 2012 (a 33% increase)
- 11 of those 20 products are categorized by the AHIP as "genetic disorders"

New learnings - fresh for 2017

1. Products approved by the FDA, but deemed by the insurer to be "Investigational and not Medically Necessary." An unofficial threshold of $200,000 per year per patient may be emerging. Case examples:
   - EXONDYS by Sarepta for DMD. Anthem's policy, effective 3/29/17, does not cover EXONDYS for DMD
   - SPINRAZA by Biogen for SMA. Humana's policy, effective 2/8/17, essentially limits use to infantile SMA1

2. Increasingly long P.A. restrictions. On the aforementioned brands, UHCs policies (effective 4/1/17 and 5/1/17) have 6-8 criteria for initial therapy (vs. 1-3 for "classical" ultra-orphan brands). Increased restrictions, whether by UHC or others, include:
   - Restricting use to patients precisely matching Inclusion Criteria from clinical trials
   - More aggressive perspective on the "efficacy & safety" issues that arose in the trial
   - Requiring increased documentation of genetic screening, e.g., the gene copy number variation (CNV)
   - Requiring more frequent reporting of patient outcomes, e.g., ensuring that patient outcomes, within 3 months, achieve outcomes as positive as in clinical trial results

Qral Group conducts quarterly roundtables, please contact us to book a two hour session to focus on your products.

Adam Hadjinian
San Francisco
ahadjinian@qralgroup.com
415 347 9565

Patrick Kennedy
Chicago
pkennedy@qralgroup.com
708 347 0077

Ying Li
New York
yli@qralgroup.com
646 740 7369

Sources: Research reports: Leerink Swan, LifeSci Capital. "High-Priced Drugs Report" by AHIP. Medical policies: payer websites. Exondys, Sprinraza (x,y): CDC, OrphaNet.