Converge Advisory Group

Glybera, Spinraza, Exondys…oh my!!

Innovative rare disease therapies and their rocky commercial starts

	Glybera (UniQure)	**Spinraza (Biogen, Ionis)**	**Exondys 51 (Sarepta)**
The Basics	treats ultra-rare familial lipoprotein lipase deficiency. Manufacturer confirmed poor sales (1 patient as of May 2016) and will not be renewing EU application. Annual Cost of Therapy: >$1M Tx Type: Adenovirus w/good gene	treats spinal muscular atrophy. Approved in Dec 2016, and had three payers restricting access, for only Type 1, a much smaller population of living patients. Annual cost: $700K + ($375K x 3yrs) Tx Type: Antisense Oligonucleotide	treats Duchenne Muscular Dystrophy. Insurers have stringent reimbursement criteria, including a walking score, even though many patients can't walk. Annual cost: $300K Tx Type: Antisense oligonucleotide
The Debate	The one patient, who was reimbursed in the 4 years since launch was hospitalized 40 times before therapy, and no ER visits afterwards. Doctor had to submit a 'thesis' to German insurer. Clinical studies showed safety and efficacy. Payers were discussing parlaying reimbursement over time: staggering price, not enough patients.	Within 3 months of approval: three insurers placed reimbursement limitations. Anthem initially deemed it 'not medically necessary' for Types 2-4, requiring pay-for-performance package, reviews every 6 months, documenting clinical improvements. Upside: 1Q2017 sales surpassed expectations; EU approval imminent	Lots of controversy on FDA approval: advisory committee didn't approve, CBER director overruled. Anthem didn't deem as medically necessary one month post-approval. Bounced back so far: payers having more positive opinion, treating 250 patients.

As rare disease and cell therapies get approved and launched, the traditional drug development pathway isn't Fit for Purpose for all therapies. There appears to be a big gap when translating clinical results to reimbursable evidence.

Do payers have a material role in development? How do we better translate clinical results to a marketed drug?

Does price need to be set in stone at launch? Can elastic price-value time-bound parameters be applied?

How do we manage hope for patients? What does this say about us as healthcare professionals?

*Imagine you were a patient/parent and couldn't get the **ONLY** **APPROVED** drug!*