

Spinraza Access Webinar

February 14, 2017



Make today a breakthrough.

After Approval - Access

- Not Unexpected Issues:
 - 2016 Conference slide.
 - **Sites and capacity.**
 - **Insurance coverage.**
- But – earlier and broader approval:
 - Trials stopped early – interim.
 - 4 months before official FDA deadline.
 - All ages and all types, no restrictions:
 - FDA did more than usual (extrapolated).
- Rules and Regulations:
 - Who can say what.

2016 Conference - After Approval...

- **Proving it – the first step to broad access...**
 - **Make it.**
 - **Deliver it.**
 - **Pay for it.**
 - **Expand it.**

Access Metrics

- General summary, what we're hearing:
 - All types have been approved/dosed.
 - Ages from a few weeks old to late 30's.
 - In 14 states.
 - Not heard of specific insurance denials.

Drug Pricing – Orphan Diseases

- Incentive to invest:
 - High research costs and risks.
 - Encourages pharma not to ignore.
- Smaller patient numbers:
 - Higher price.
 - List price of \$750,000 in first year, \$375,000 each year after.
- From value:
 - How well does it work.
 - Save and extend and improve life.
 - To patient and family and society.

Drug Pricing – Orphan Diseases

Disease	Total US Population	Drug	Annual List Price	On Therapy	Impact - Competition	Value on Therapy
Pompe	4,000	Myozyme/ Lumizyme	\$200,000	3,500	Stop/safety – first	\$0.7B
SMA	10,000	Spinraza	\$450,000	5,000	Stop – first	\$2.3B
CF	30,000	Orkambi/ Kalydeco	\$275,000	9,000	Slow – first	\$2.5B
Hep C	3,000,000	Solvaldi/ Harvoni	\$90,000	200,000	Reverse – several	\$18.0B
High cholesterol	78,000,000	Statins	\$150	43,000,000	Slow – many	\$6.5B

Insurance

- Variable decisions:
 - Many groups.
 - Commercial and Medicaid.
- More data needed:
 - From current trials, interim, publication.
 - Other untested areas needed:
 - Adults and fusion/rods
- Ongoing proof.
- Drug cost and procedure cost.
- Don't get discouraged:
 - Denials:
 - Let us know – Consolidate reasons (data vs price).
 - Let Biogen know - Support programs.

Sites and Capacity

- Currently have ~20 to 30 sites in US:
 - Trial sites, and care centers of excellence.
 - Already maxed out:
 - Need new sites.
- Need potentially ~200 to 300 sites:
 - Loading dose period.
 - Complicated procedure:
 - SMA care important.
 - ~25 to 50 patients at each site per year, staggered.
 - Adult and pediatric
 - Others specializations
 - In-state
- Training and education:
 - On SMA.
 - On drug and procedure.
 - Role for the community.

New Sites - List

- Progress since start of year:
 - 237 total sites contacted.
 - 80 sites now being communicated with.
 - 25 sites on list confirmed.
 - 18 states with at least one site.
- Expect waiting lists.
- Some will specialize, prioritize, randomize.
- List resources when ~80% of states with at least 1 site.

Insurance Coverage

	Anthem	United Health	Humana	Aetna	Cigna	Medicaid (By state)
Type 0	Yes	No	Yes			
Type I	Yes	Yes	Yes			
Type II	Uncertain	Yes	Uncertain			
Type III	No	Yes	No			
Type IV	No	Uncertain	No			
1 SMN2	Yes	No	Yes			
2 SMN2	Yes	Yes	Yes			
3 SMN2	No	Yes	No			
4 SMN2	No	Yes	No			
Pediatric	Yes	Yes	Yes			
Adult	Uncertain	Yes	Uncertain			
Trach	Yes	No	Yes			
Non-invasive 6	Yes	No	Yes			

Insurance Coverage

- Resources:
 - Current policies:
 - Visit: www.cureSMA.org/Spinraza
 - Find: Resources → Insurance Policies
 - *Choice and Connection to Care: A Health Insurance Roadmap for People Living with Spinal Muscular Atrophy (SMA) and Their Caregivers*
 - A new Cure SMA care series booklet on navigating commercial and government health insurance options.
 - Projected release date: March 1, 2017
 - Watch: www.cureSMA.org/news for an official announcement

Next Steps

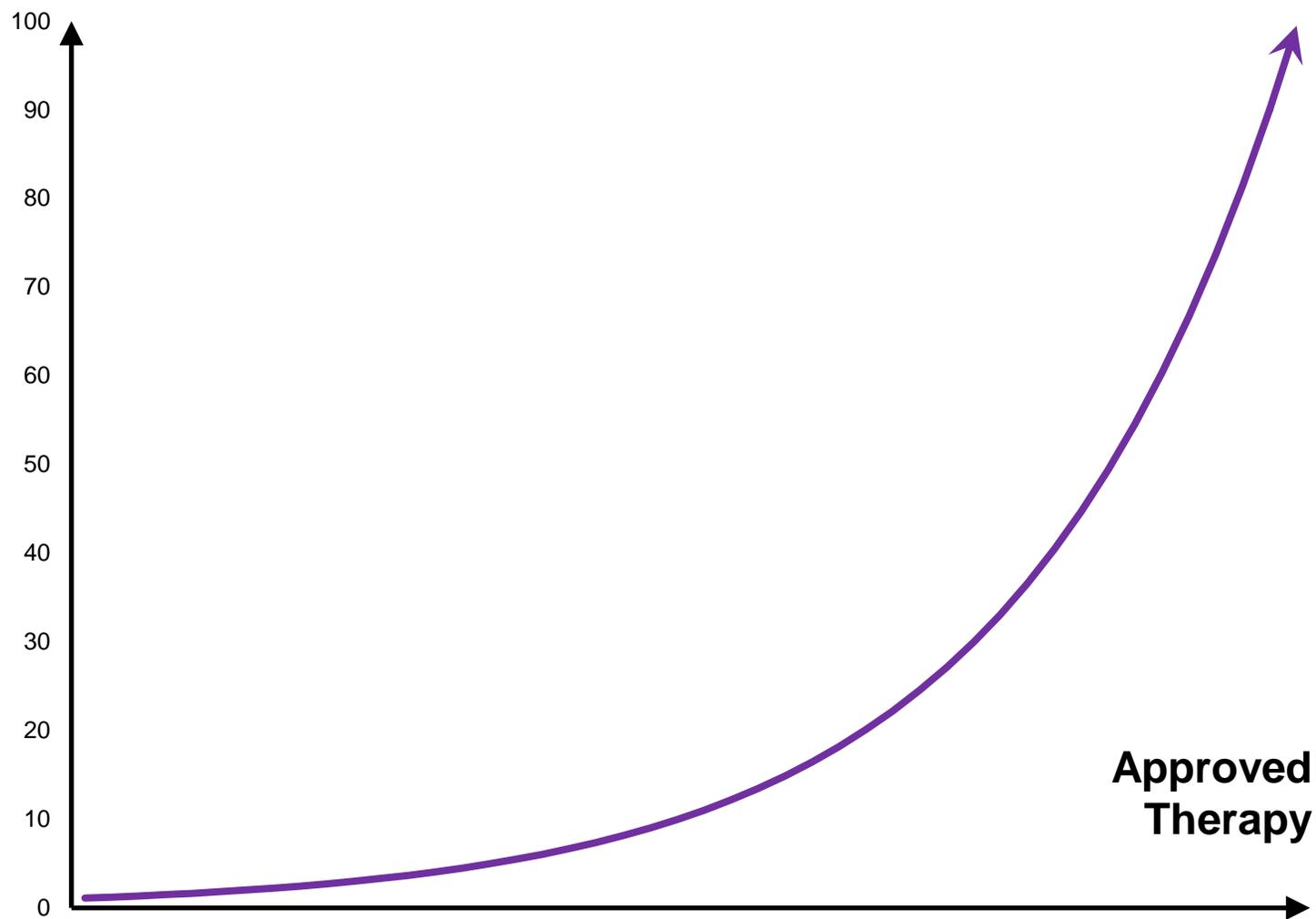
- Get involved:
 - Send site contacts to patientcare@curesma.org
 - Community survey – live now:
www.cureSMA.org/communitysurvey
 - Potentially additional trials and data collection.
- Care centers program, and new site training and education.
- Resources:
 - Site list

Next Steps

- Coverage & Payment Policy Project: The goal of the Coverage and Payment Policy Project is to help facilitate a favorable public and commercial coverage and payment environment for the full range of care and treatment for people with all types of SMA of all ages. The project involves three major components:
 - Develop materials and messages in support of coverage and payment for SMA care and treatment;
 - Identify and establish relationships with key public, private, and state-based stakeholders; and
 - Engage in targeted outreach to members of congress in anticipation of and following the availability of SMA treatments and in preparation for future engagement with policymakers to support favorable coverage and payment for SMA care and treatment.
- Biogen community support resources: for questions on your specific insurance/copay situation.
 - Visit: www.spinraza.com
 - Find: Support Services

The Closer We Get.....

Level of Frustration



Different Levels of Access

General Items

- No information yet on what happens if stop taking.
- Takes time to work, and other care important for best impact.
- Ongoing monitoring.
- Other therapies – trials or approved.

- We do not have as much data on Spinraza's effects in adults as we do in children, but we do have results that show it improves nerve function in patients with all types of SMA (SMA1, SMA2 and SMA3) who are aged 0-20 years of age. Because of the dramatic effects of Spinraza seen in tissue from patients, as well as its dramatic and consistent effects in patients with all types of SMA, the FDA approved the drug for all patients with SMA without any restrictions regarding the type of SMA, the age of the patient or the severity of the patient's disabilities. Our primary goal in using Spinraza is always to assure that the patient's course will stabilize, and that no additional function will be lost, but in some instances there is also some degree of functional improvement.

Q & A

Thank you!