Access Webinar

September, 2017

Make today a breakthrough.

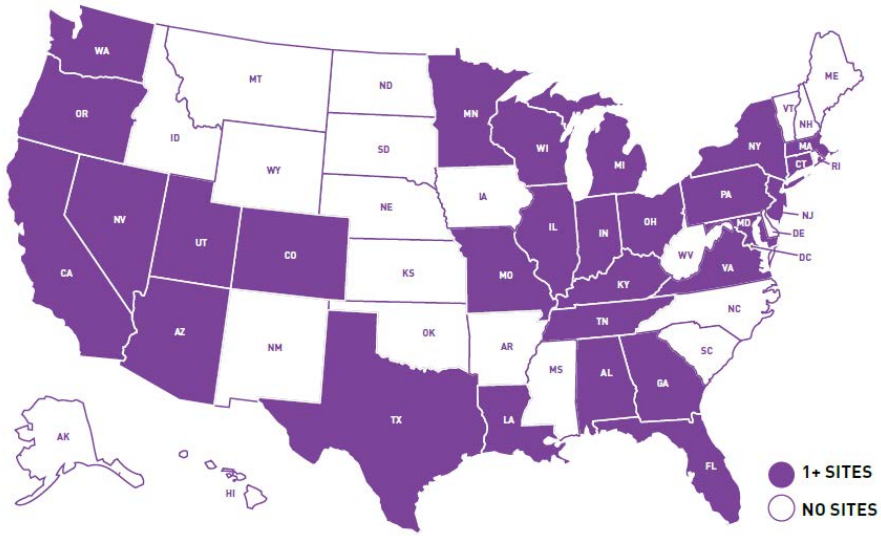
Current Dosing Metrics

- September 2017:
  - **~2,000 patients been dosed**
  - With ~250 and ~200 in trials/EAP and support programs
- All types – well balanced
- Ages from a few weeks to 50/60s
- **120 sites in 40 states have dosed** at least one patient
- 230 sites submitted a start form

100's to 1,000's

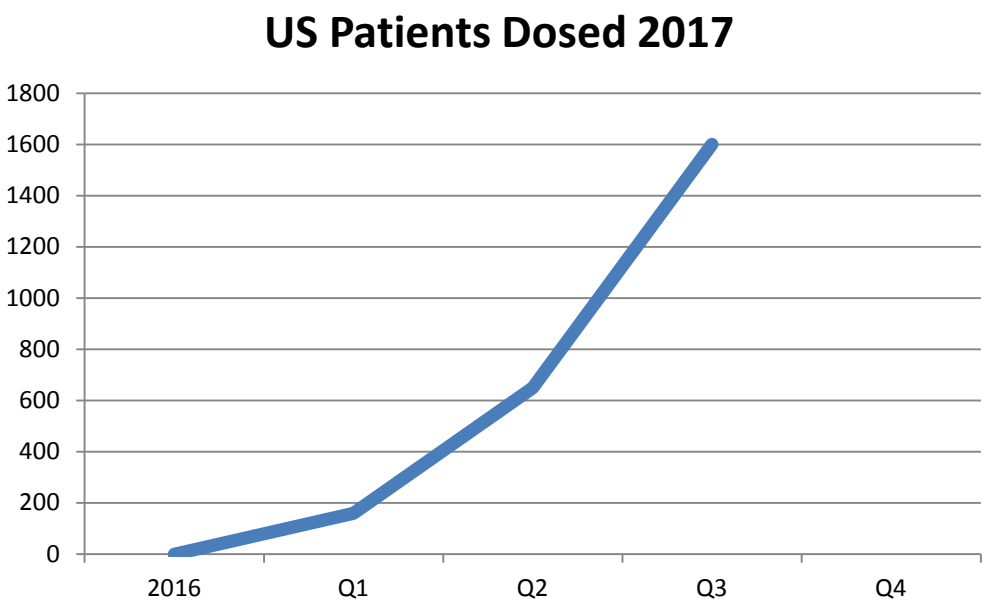
Cure SMA List – New Sites

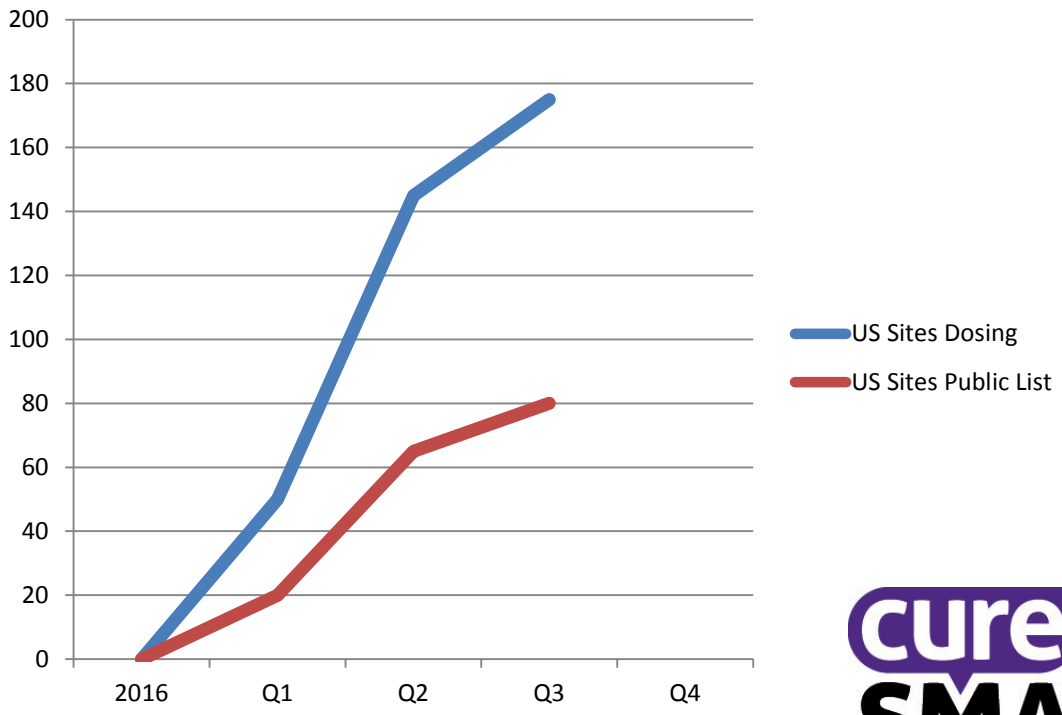
- Progress since start of year:
  - **80 sites on list confirmed.**
  - 177 sites being communicated with.
  - 29 states plus DC with at least one site.
  - Many sites are dosing, but not ready to publicly announce yet



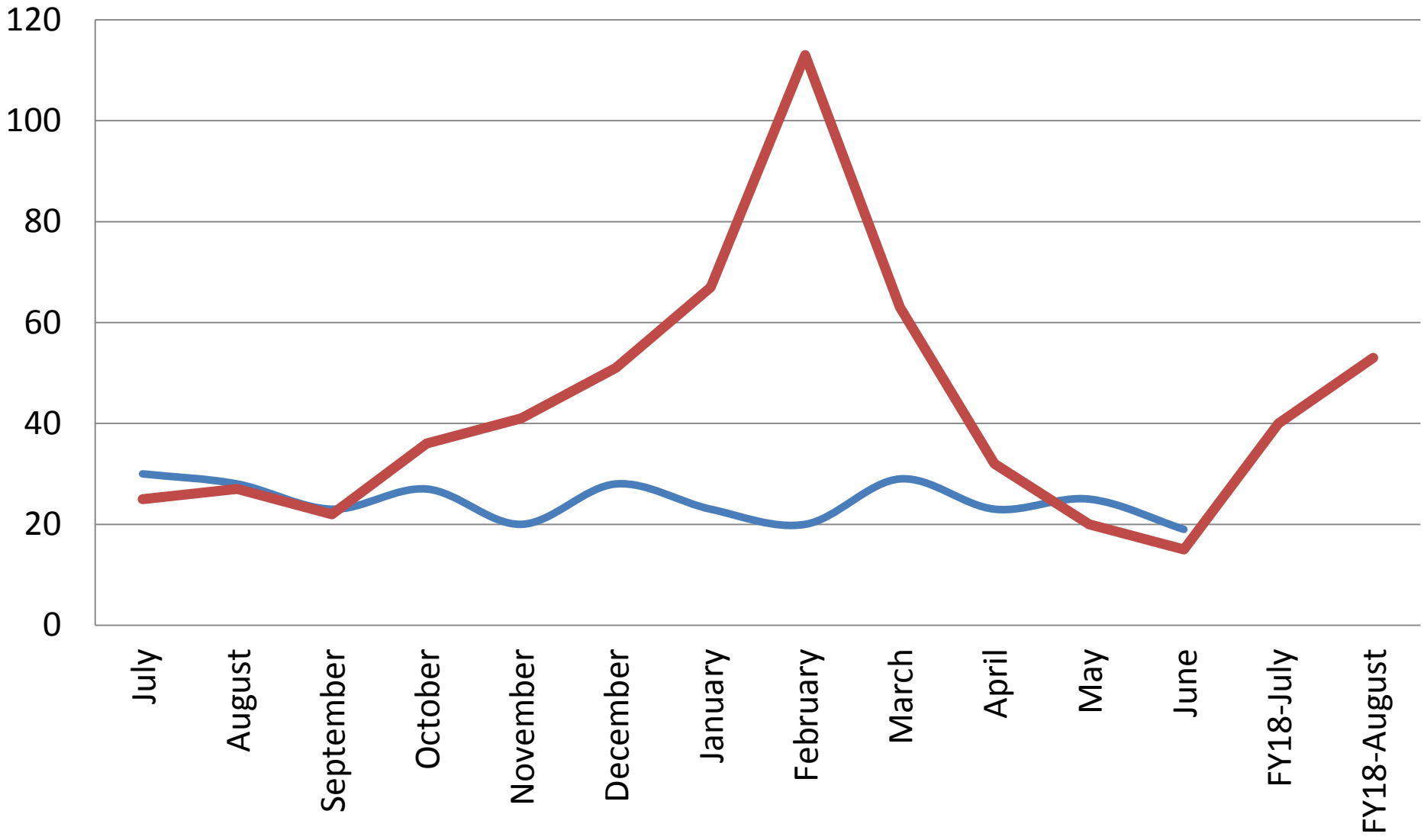
- **Need 250-300 sites to meet the needs** of our community long term:
  - Need to educate and advocate to new and pending sites
    - On SMA, drug and procedure, care
- Expect waiting lists.
  - Some will specialize, prioritize, randomize.

Progress To Date





Newly Diagnosed FY16 and FY17



Site Access Goals

-Initial Target of One Site per state

-Then at least One Adult Site and One Pediatric Site per state.

-Finally Multiple Sites per state based on population.

-Resources to sites

-Advocate to sites

Need new sites:

-Loading dose period.

-Procedure.

-~25 to 50 patients at each site per year, staggered.

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Funding to Support Treatment Access and Therapist Mentoring

- **Project Goals:**
  - Increase number of SMA patients seen, treated, and followed at sites
  - One year of funding for a maximum amount of $50,000 for total of eight sites in US

- **Project Goals:**
  - Provide resources for physical and occupational therapists new to SMA or seeking to improve the quality of care
  - Increase number of therapists who can perform motor function evaluations

Insurance Coverage Status

- Variable rules and decisions:
  - Commercial and Medicaid and Medicare
- More data needed:
  - Publications on existing data:
    - Trach, Trials.
  - Untested areas – need to collect:
    - Fusion, Older.
- Ongoing proof:
  - 6 months to annually to longer
  - Individual data vs historical averages

Don't get discouraged:
Policies are not fixed:
Updated and individually implemented

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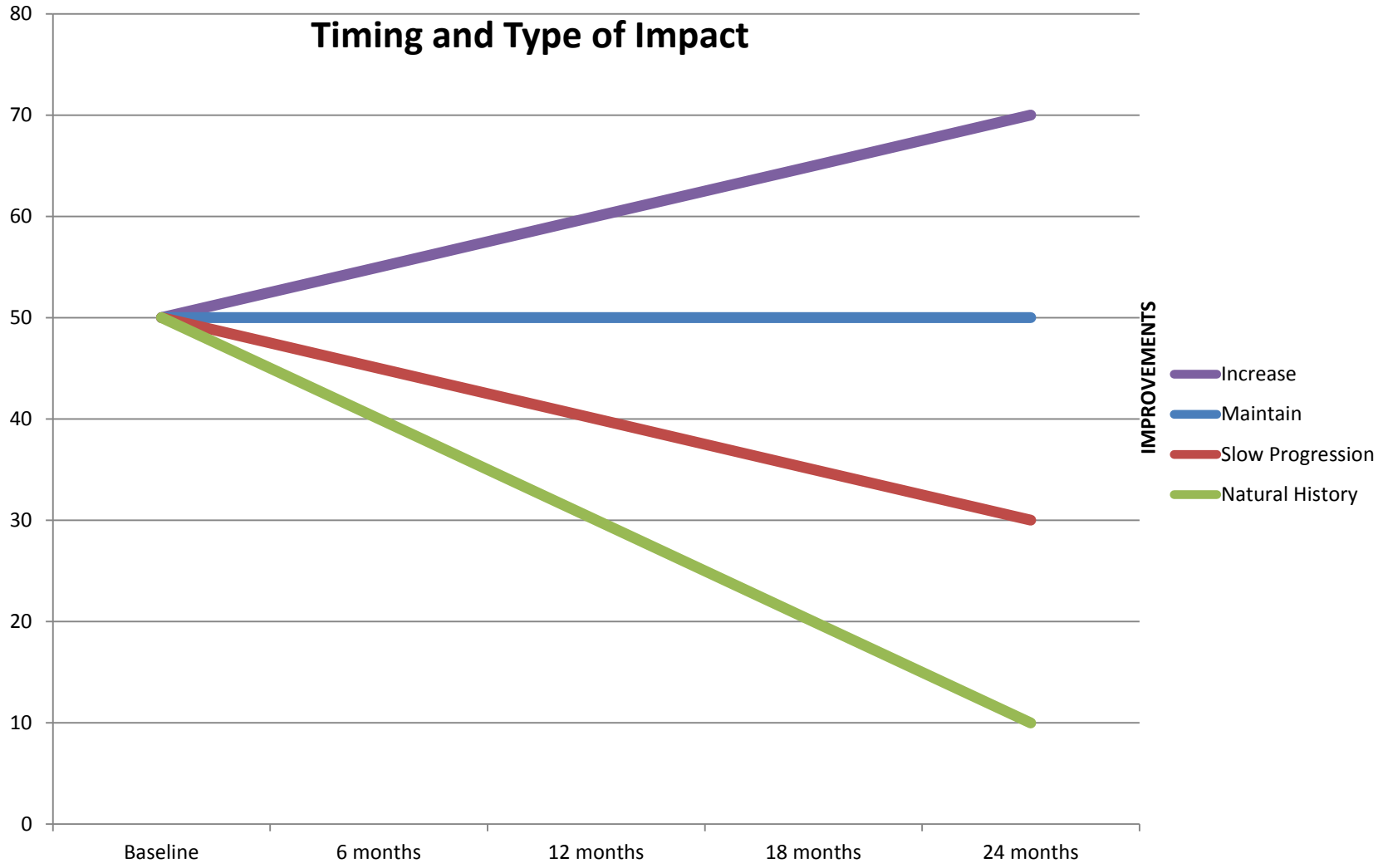
Commercial Coverage

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

Medicaid Coverage

	CA	DE	FL	DE, IA, MN, NC, NV, WA	MI	OR	MO	NE	PA	SD	WI
Type 0	No	No	No	Yes	No	Yes	No	No	No	No	No
Type I	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes
Type II	Yes	Yes	Yes	Yes	No	Yes	Yes	Yes	Yes	Yes	Yes
Type III	Yes	Yes	Uncertain	Yes	No	Yes	Yes	Yes	Yes	Yes	Yes
Type IV	No	No	No	Yes	No	Yes	Uncertain	No	No	No	No
1 SMN2	Yes	Yes	No	Yes	No	Yes	No	No	Yes	Yes	No
2 SMN2	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes
3 SMN2	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	No	Yes
4 SMN2	Yes	Uncertain	No	Yes	Yes	Yes	Yes	Yes	Yes	No	Yes
Pediatric	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes
Adult	Yes	Yes	Yes	Yes	Uncertain	Yes	Yes	No	Yes	Uncertain	No
Trach	Yes	Yes	No	Yes	No	No	Yes	Yes	Yes	No	No
Non-invasive	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	Yes	No	No

Impact


Timing and Type of Impact
80
70
60
50
40
30
20
10
0
Baseline
6 months
12 months
18 months
24 months
IMPROVEMENTS
Increase
Maintain
Slow Progression
Natural History

Clinical Trial Data: What to expect?

- ENDEAR: 51% of treated infants gained motor milestones vs. 0% of untreated group
  - An example of a motor milestone gain would be moving from unable to roll to rolling onto side, or moving from unable to sit to sitting with support.
- ENDEAR: 61% of treated infants were alive and did not require permanent ventilation vs. 32% of untreated infants.
- ENDEAR: motor function gains in infants on permanent ventilation to be presented at World Muscle Society Meeting in October
- EMBRACE: First data to be presented at World Muscle Society in October which included older type I patients
- Early Access Program: 1st experience report at World Muscle Society
- CHERISH: Gain of 4 HFMSE points in treatment group vs. loss of 1 HFMSE point in untreated group
- No data yet in older teens and adults
- Quick vs. Build up

Renewals Reauthorizations

- Drug is given enough time to work.
  - by building up the muscle over time
  - would expect that this will be taking place over a year or two rather than months.
- The measurements of impact are being made as a comparison against what would have happened without the drug.
  - In SMA, patients decline and go downhill in their function over time (some more rapidly than others).
  - An impact from a drug is anything better than this.
  - Which could be improving (now going up) or maintaining (staying stable and keeping functions) or even a decline but at a slower than normal/usual/expected rate.
- New area just starting now
- Keep your own records – notes and videos.
- Risk benefit survey
- Annual membership update

Cure SMA Coverage & Payment Activities

- **Letters to CEOs and CMOs of all major insurance plans and companies and to all of the state Medicaid directors and governors:**
  - urging coverage of Spinraza for all ages and types
  - Moving in right direction….
- **Meetings with various national organizations and government agencies**

- Current policies and all data **www.cureSMA.org/Spinraza**
  - Choice and Connection to Care: A Health Insurance Roadmap for People Living with Spinal Muscular Atrophy (SMA) and Their Caregivers
  - Know Your Resources: How Congressional Caseworkers Can Help You Obtain Insurance Coverage for New Therapies

Medicare Advocacy

- Medicare's process for new drugs, like Spinraza, is different from Medicaid's approach.
  - Medicaid issues a policy up front that guides whether individual claims are approved or denied.
  - In Medicare, the process begins with individual claims being reviewed on a case-by-case basis until a consensus emerges. Once that happens, local policies are issued, potentially followed by a national policy.

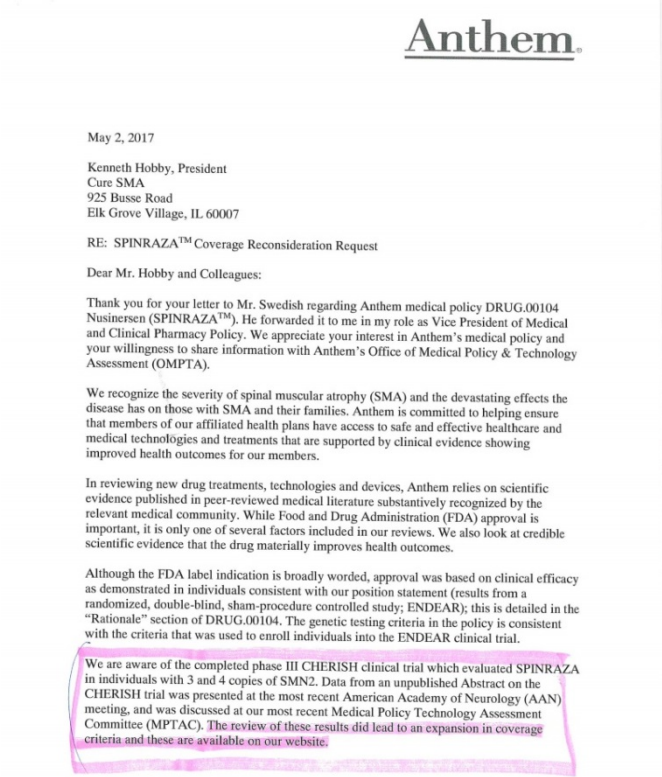
- At a meeting with Cure SMA staff, the leadership of CMS asked for our community's help and guidance in this process.
  - We've launched survey collecting data to help CMS assess whether to accelerate the process of issuing a national policy, or whether to continue to provide support as cases are reviewed at the local level.

- If you are an individual affected by SMA with Medicare coverage, or if you care for an individual affected by SMA on Medicare, please take this short survey by Friday, September 29: **events.curesma.org/medicare**

Key Advocacy Items

- Same approach as with FDA:
  - Doctors, Hospitals, Insurance.
- Proactive positive patient voice
  - Stay motivated
- Appeals
- Individual and for community
  - Precedents
  - Advocate on data and need.

SMA is one disease (not types):
-Same genetic cause and target SMN1 and SMN2
-All have genetic target of Spinraza
Clinical Significant Impact:
-Trial data, broad and positive and significant
Urgent and critical and unmet need
Patient Meaningful Impact:
-Same as to the FDA
-Slow or stop progression or incremental changes



Anthem

May 2, 2017

Kenneth Hobby, President
Cure SMA
925 Busse Road
Elk Grove Village, IL 60007

RE: SPINRAZA™ Coverage Reconsideration Request

Dear Mr. Hobby and Colleagues:

Thank you for your letter to Mr. Swedish regarding Anthem medical policy DRUG.00104 Nusinersen (SPINRAZA™). He forwarded it to me in my role as Vice President of Medical and Clinical Pharmacy Policy. We appreciate your interest in Anthem's medical policy and your willingness to share information with Anthem's Office of Medical Policy & Technology Assessment (OMPTA).

We recognize the severity of spinal muscular atrophy (SMA) and the devastating effects the disease has on those with SMA and their families. Anthem is committed to helping ensure that members of our affiliated health plans have access to safe and effective healthcare and medical technologies and treatments that are supported by clinical evidence showing improved health outcomes for our members.

In reviewing new drug treatments, technologies and devices, Anthem relies on scientific evidence published in peer-reviewed medical literature substantively recognized by the relevant medical community. While Food and Drug Administration (FDA) approval is important, it is only one of several factors included in our reviews. We also look at credible scientific evidence that the drug materially improves health outcomes.

Although the FDA label indication is broadly worded, approval was based on clinical efficacy as demonstrated in individuals consistent with our position statement (results from a randomized, double-blind, sham-procedure controlled study; ENDEAR); this is detailed in the "Rationale" section of DRUG.00104. The genetic testing criteria in the policy is consistent with the criteria that was used to enroll individuals into the ENDEAR clinical trial.

We are aware of the completed phase III CHERISH clinical trial which evaluated SPINRAZA in individuals with 3 and 4 copies of SMN2. Data from an unpublished Abstract on the CHERISH trial was presented at the most recent American Academy of Neurology (AAN) meeting, and was discussed at our most recent Medical Policy Technology Assessment Committee (MPTAC). The review of these results did lead to an expansion in coverage criteria and these are available on our website.

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Current US Trials

- A Study of CK-2127107 in Patients With Spinal Muscular Atrophy (Clinicaltrials.gov Identifier: NCT02644668)
  - General Criteria*: Teens & adults 12 years of age or older (ambulatory and non-ambulatory) with SMA type II, III or IV.
  - Phase: Phase 2

- A Study to Investigate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics and Efficacy of RO7034067 in Infants with Type 1 Spinal Muscular Atrophy (Clinicaltrials.gov Identifier: NCT02913482)
  - Also Known By: FIREFISH
  - General Criteria*: Infants age 1-7 months with SMA type I.
  - Phase: Phase 2

- A Study to Investigate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics and Efficacy of RO7034067 in Type 2 and 3 Spinal Muscular Atrophy Participants (Clinicaltrials.gov Identifier: NCT02908685)
  - Also Known By: SUNFISH
  - General Criteria*: Children, teens and adults age 2-25 with SMA type II or III.
  - Phase: Phase 2

- A Study of RO7034067 in Adult and Pediatric Participants with Spinal Muscular Atrophy (Clinicaltrials.gov Identifier: NCT03032172)
  - Also Known By: JEWELFISH
  - General Criteria*: Children, teens and adults age 12-60 who have previously been exposed to an SMN2-targeting therapy.
  - Phase: Phase 2

Considerations on Approved Treatment vs. Trials

- **Personal decision dependent on many factors**
- **Important to discuss those factors with your neurologists**
  - Evaluate available safety and efficacy data
  - Evaluate route and timing of administration
  - Understand inclusion criteria for trials
  - Understand exclusion criteria for trials:
    - Whether previous drug exposure allowed and when
    - **Most trials today will prohibit co-utilization with Spinraza**
- **Recommend not waiting for future trials to open, if other options now**
  - Delays in opening trials are very common and can't be predicated
  - Changes in inclusion and exclusion are very common until formally announced
  - Earliest possible administration of SMN enhancing drugs will yield best results

Timing is one of biggest factor in degree of response to SMN

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International Approvals

- Spinraza is now approved in:
  - US
  - EU
  - Japan
  - Canada
  - Brazil
- Marketing authorization applications for Spinraza are currently under review in:
  - Switzerland
  - Israel
  - South Korea
  - Australia
  - Additional international filings are also being planned.

Tips for Initial Appointments

- Fill out your portion of the Start Form and bring it with you to your appointment
- Bring your insurance cards and make sure the office submits copies of your insurance cards with the Start Form
- Have the office submit your clinical records with the Start Form
- Have the office submit a Prescription with your Start Form (preferably for the 4 loading doses and 3 maintenance doses); make sure they sign and date the RX.
- Ask them if you can get a genetics test done during your appointment or if you can talk to the genetic counselor to get the kit ordered.
- Talk to them about baseline strength testing and getting that started.
- If you have rods- get an x-ray or CT scan done to see if there is space to administer the lumbar puncture.

Q & A

Thank you!