



SPINRAZA (nusinersen): Setting The Standard For Dynamic Collaboration

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BIOGEN



We are led by great science

Committed to areas of high unmet need

Opening up new therapeutic options like Spinraza

A STORY OF COLLABORATION

BIOGEN

We saw an unmet need

PATIENTS

Guided our path

FDA

Made it a reality

A Relationship Of Mutual Respect

SPINRAZA



SMA

Spinraza: A Case Study

PATIENTS ARE OUR SMEs



Partners in research

Advisors in development

Most important stakeholders

OUR FOUNDATION

**Do it right
the first time**

- 1 Partner with the patient community
- 2 Follow the science
- 3 Start with rigorous trial design
- 4 Employ flexibility in trial design and analysis
- 5 Use FDA's expedited programs – Priority Review and Fast Track

WHAT WE LEARNED

Effective Communication + Collaboration = Speed

WE ARE JUST GETTING STARTED



PRESYMPTOMATIC SMA

SYMPTOMATIC – INFANTILE-ONSET SMA

SYMPTOMATIC – LATER-ONSET SMA

NURTURE PRIMARY ENDPOINT: NO DEATHS OR CHRONIC RESPIRATORY SUPPORT AMONG SMA PATIENTS TREATED IN PRE-SYMPTOMATIC PERIOD

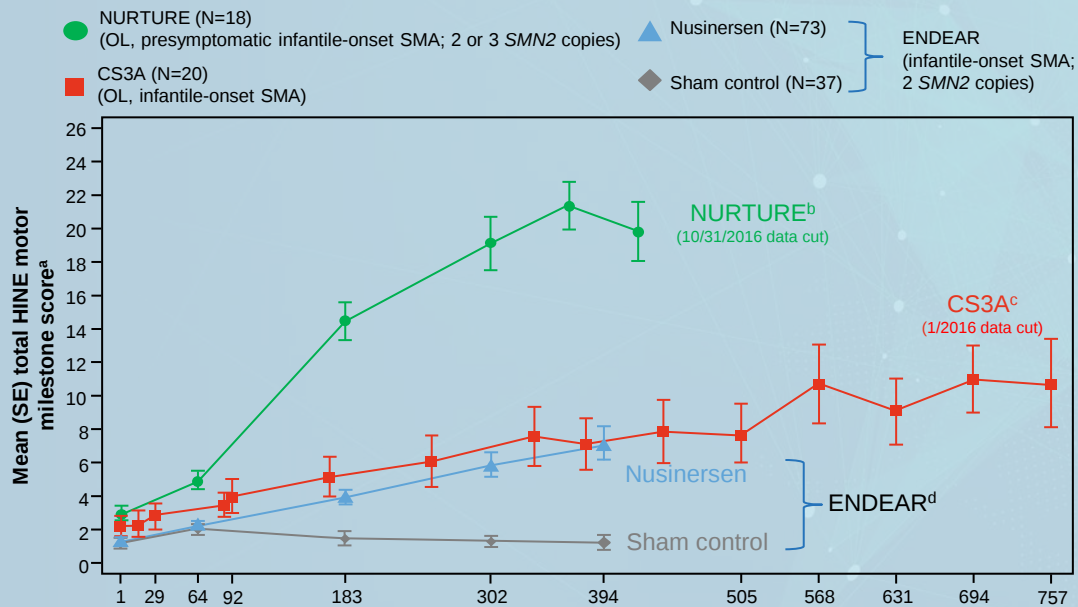
At the time of the interim analysis, infants had been on study for a median (range) of 317.5 (2–524) days

All infants were alive and none had required respiratory intervention^a

Nusinersen-treated infants, n (%)	2 SMN2 copies n=13	3 SMN2 copies n=7	Total n=20
Alive	13 (100)	7 (100)	20 (100)
Required invasive ventilation or tracheostomy	0	0	0
Required noninvasive ventilation for ≥6 hours/day continuously for ≥7 days	0	0	0

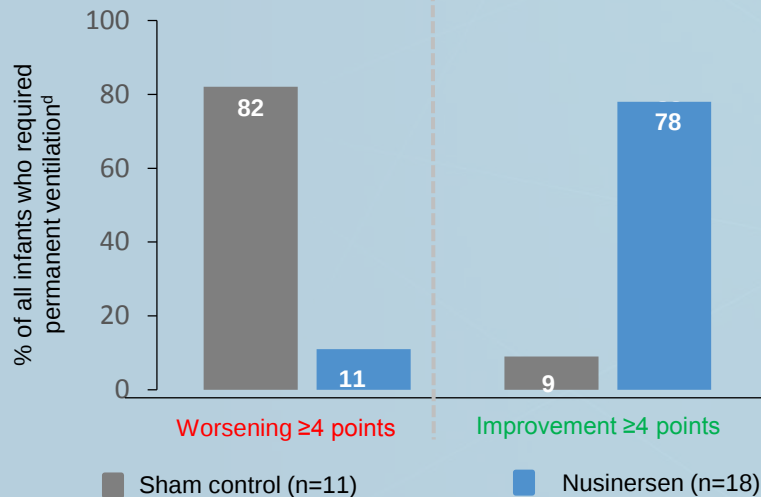
NURTURE study interim analysis data cut-off date: October 31, 2016. ^aRespiratory intervention was defined as invasive or noninvasive ventilation for ≥6 hours/day continuously for ≥7 days or tracheostomy.

IMPROVEMENTS IN MOTOR MILESTONE SCORES ACROSS STUDIES OF PRE-SYMPTOMATIC AND SYMPTOMATIC SMA INFANTS



IMPROVEMENT IN MOTOR FUNCTION AMONG SMA INFANTS TREATED AFTER SYMPTOM ONSET AND REQUIRE PERMANENT VENTILATION

Total CHOP-INTEND score change from baseline^c



- Nusinersen-treated infants demonstrated more improvement and less worsening in motor function.^b



OUR FUTURE, TOGETHER

We are putting the best practices in
place to speed our efforts to bring
transformative therapies to patients with
few or no treatment options