

# SEATTLE CHILDREN'S HOSPITAL

Department of Pediatric Neurology  
4800 Sand Point Way NE | Seattle, WA 98105

## NEUROLOGY ASSESSMENT

Patient:	Aiden Foster
DOB:	2022-03-10
Age:	3 years
MRN:	AIDEN_F
Assessment Date:	2026-01-25
Evaluating Physician:	Dr. Amanda Liu, MD

### CHIEF COMPLAINT

SMA Type 2 with declining motor function despite prior Zolgensma gene therapy — requesting Spinraza as rescue/add-on therapy

### HISTORY OF PRESENT ILLNESS

3-year-old male with SMA Type 2 (homozygous SMN1 deletion, 3 SMN2 copies) diagnosed at 9 months when motor milestones plateaued (could sit but never pulled to stand). Received Zolgensma at 11 months with initial improvement — gained sitting endurance and some upper limb function (HFMSE 14 to 24). Motor function stable for approximately 12 months post-gene therapy. Over the past year, progressive decline observed: HFMSE dropped from 24 to 18, decreased upper extremity reach, new nocturnal hypoventilation requiring BiPAP. Anti-AAV9 antibody titers are high (1:102,400), precluding Zolgensma re-dosing. Risdiplam was considered but parents prefer intrathecal Spinraza based on published data showing benefit in post-gene-therapy patients. Prescribing neurologist believes Spinraza could stabilize or slow decline given its different mechanism of action (antisense oligonucleotide vs. gene replacement).

### MOTOR FUNCTION ASSESSMENT

SMA Type:	Type 2 (intermediate)
Motor Milestones:	Can sit independently but has never walked. Progressive decline in upper extremity function over past 12 months.
Current Motor Function:	Sits independently. Cannot stand or walk. Moderate upper limb weakness — difficulty raising arms above shoulder height. Fine motor skills declining. HFMSE score 18 (down from 24 one year ago).
HFMSE Score:	18 (prior: 24, decline: 6 points)
Feeding Status:	Oral feeding with soft diet. Occasional coughing with thin liquids.
Respiratory Status:	Mild restrictive pattern on PFTs. Uses BiPAP nocturnally. Not on permanent ventilator.
Ventilator Dependent:	No

### ASSESSMENT AND PLAN

**Rationale for Spinraza:** Gene therapy effect appears to be waning after 2+ years. Spinraza acts via a distinct mechanism (SMN2 pre-mRNA splicing modification) and could complement the residual SMN protein produced by Zolgensma. Published case series and the RESPOND trial data suggest benefit of nusinersen in post-gene-therapy patients with declining motor function.

Recommend initiation of Spinraza (Nusinersen) therapy based on clinical assessment.

Dr. Amanda Liu, MD

Pediatric Neurology

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Date: 01/25/2026