

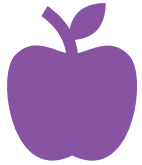


SPINAL MUSCULAR ATROPHY: AN INTERPROFESSIONAL APPROACH

Provided by
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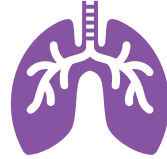
 **RMEI** MEDICAL EDUCATION
FOR BETTER OUTCOMES

Biomechanical Complications of SMA



Nutritional

- Chewing
- Swallowing
- Failure to thrive
- Failure to gain weight



Pulmonary/Respiratory

- Breathing
- Difficult to cough forcefully
- Sleep disordered breathing
- Respiratory infections



Musculoskeletal

- Muscle weakness
- Difficulty moving
- Difficulty meeting milestones
- Motor skill development
- Contracture
- Scoliosis

Nursing Considerations

Nurses need to work with other members of the team to make sure the treatment plan being developed by the team actually works for the patient.

For example:

- *Does the patient have everything at home to carry out physical therapy recommendations?*
- *If they are getting a wheelchair, is their home wheelchair accessible?*
- *Do they have what they need to be functioning members of their family and community?*

Goal of the Nurse

Align all interventions needed for the treatment of SMA to a patient's life:

- Make sure interventions work for the individual and their family
- Make sure patients have what they need to accommodate those interventions

Coordinating Care: Simple Steps



Dedicated
Person



Real-time
Collaboration



Outside
Relationships

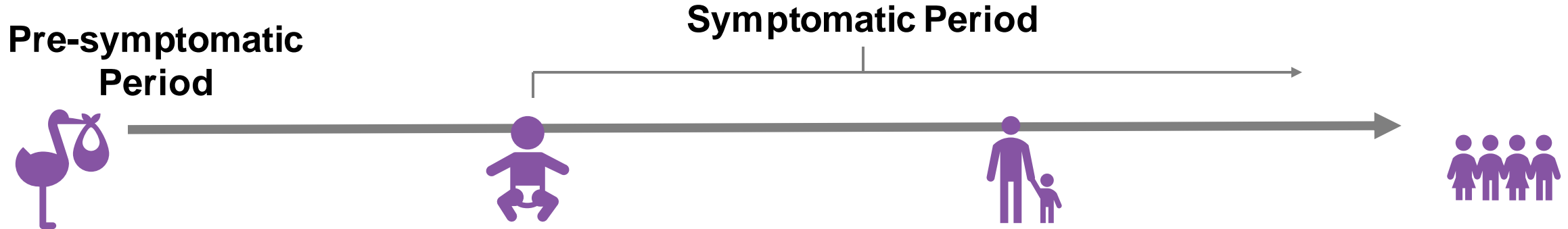
Guidelines recommend institutions coordinate all multidisciplinary assessments and visits for families, and not leave the families to navigate on their own.

Disease Modifying Therapy

	Nusinersen	Onasemnogene Apeparvovec	Risdiplam
Approval	2016	2019	2020
Mechanism of Action	SMN2-directed antisense oligonucleotide	Adeno-associated virus vector-based gene therapy	SMN2 splicing modifier
Indication	SMA in pediatric and adult patients	Patients <2 years of age with SMA with bi-allelic mutations in the SMN1 gene	SMA in patients ≥ 2 months of age
Route	Intrathecal	IV	PO solution
Dosing Frequency	Every 4 months	One (1) lifetime dose	Daily

Nusinersin [Prescribing Information]. <https://tinyurl.com/ydprw5wk>; Onasemnogene abeparvovec [Prescribing Information]. <https://tinyurl.com/yck6shh4>; Risdiplam [Prescribing Information]. <https://tinyurl.com/bdcwcc28>

Selecting Treatment



Birth to 6 weeks	6 weeks to 6 months	6 months to <2 years	≥2 Years
<ul style="list-style-type: none"> • Age-based indications • Labs/safety considerations • Shared decision making • Treatment options (2) 	<p>Same as earlier, but now...</p> <ul style="list-style-type: none"> • Functional considerations • Nutritional status • Ventilation requirements • Hospitalized versus outpatient • Social support • Available for routine monitoring • Treatment options (2 to 3) 	<p>Same considerations as earlier:</p> <ul style="list-style-type: none"> • All 3 treatments are options 	<ul style="list-style-type: none"> • Scoliosis severity • Prior surgeries • Intrathecal access • Treatment options (2)



We will now review 2 cases demonstrating interprofessional collaboration among the healthcare team to optimize treatment and navigate complex clinical situations in SMA

Case: Too Early to Treat?



Image for illustrative purposes only.

Diagnosis

Patient is a 2-year-old who was diagnosed with type 3 SMA, 4 copies of the SMN2 gene

Case: Too Early to Treat?



Image for illustrative purposes only.

Dilemma

Parents are in denial

They refuse to affiliate with a center that has expertise and familiarity with this condition.

They chalk-up his problem as being “clumsy.”

Case: Too Early to Treat?



Image for illustrative purposes only.

Question

I emphasized to the family they need to be proactive.

How do we proceed?

Do we just wait for a further worsening of his symptoms?

My Priorities for this Family as a Nurse



Build a
Relationship

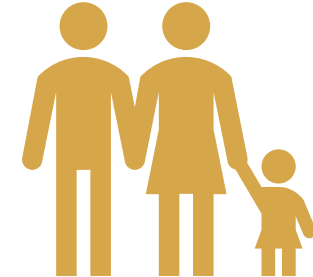
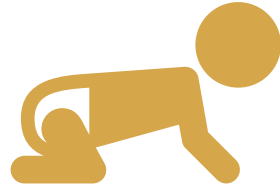


Develop Trust



Understand
Their
Perspective

My Priorities for this Family from a PM&R Perspective



Partner with
Physical
Therapy

Perform
Objective Motor
Testing

Compare
Results with
Non-SMA
Standards

Explain
Findings to
Parents

Case: Adult-Onset SMA



Image for illustrative purposes only.

Demographics

Patient is a 47-year-old man struggling with progressive loss of motor function each year

Case: Adult-Onset SMA



Image for illustrative purposes only.

Dilemma

We were able to manage his disease when he was younger, but things are getting very difficult for him. At his age, I do not know if there are any good treatment options.

He is only on supportive care/symptomatic therapy. He currently uses a BiPAP machine and requires a walker. SMN2 copy number: 4.

Case: Adult-Onset SMA

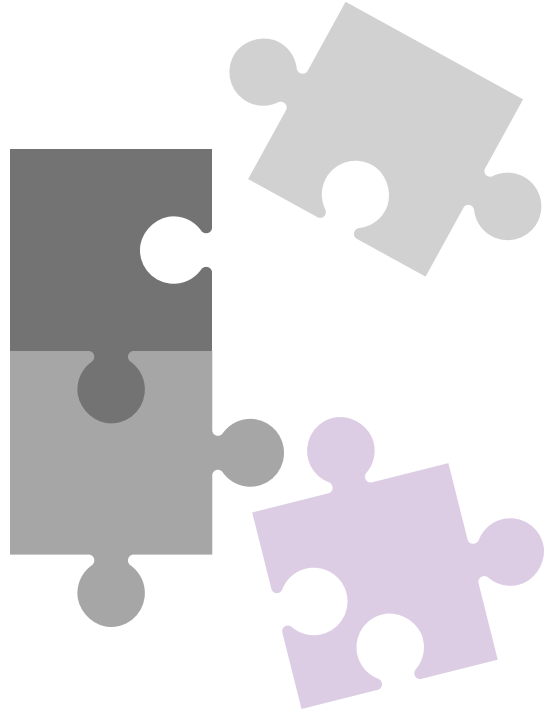


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Challenges

Adult-onset SMA is hard to recognize and very challenging to deal with as studies are mostly done in children and insurance denies disease-modifying therapies (DMTs) for adults

PM&R Considerations



Studies in Adults are Promising

Most of the studies on DMTs are in children, not adults, but recently, promising data in adults has emerged for risdiplam and nusinersen¹⁻⁷

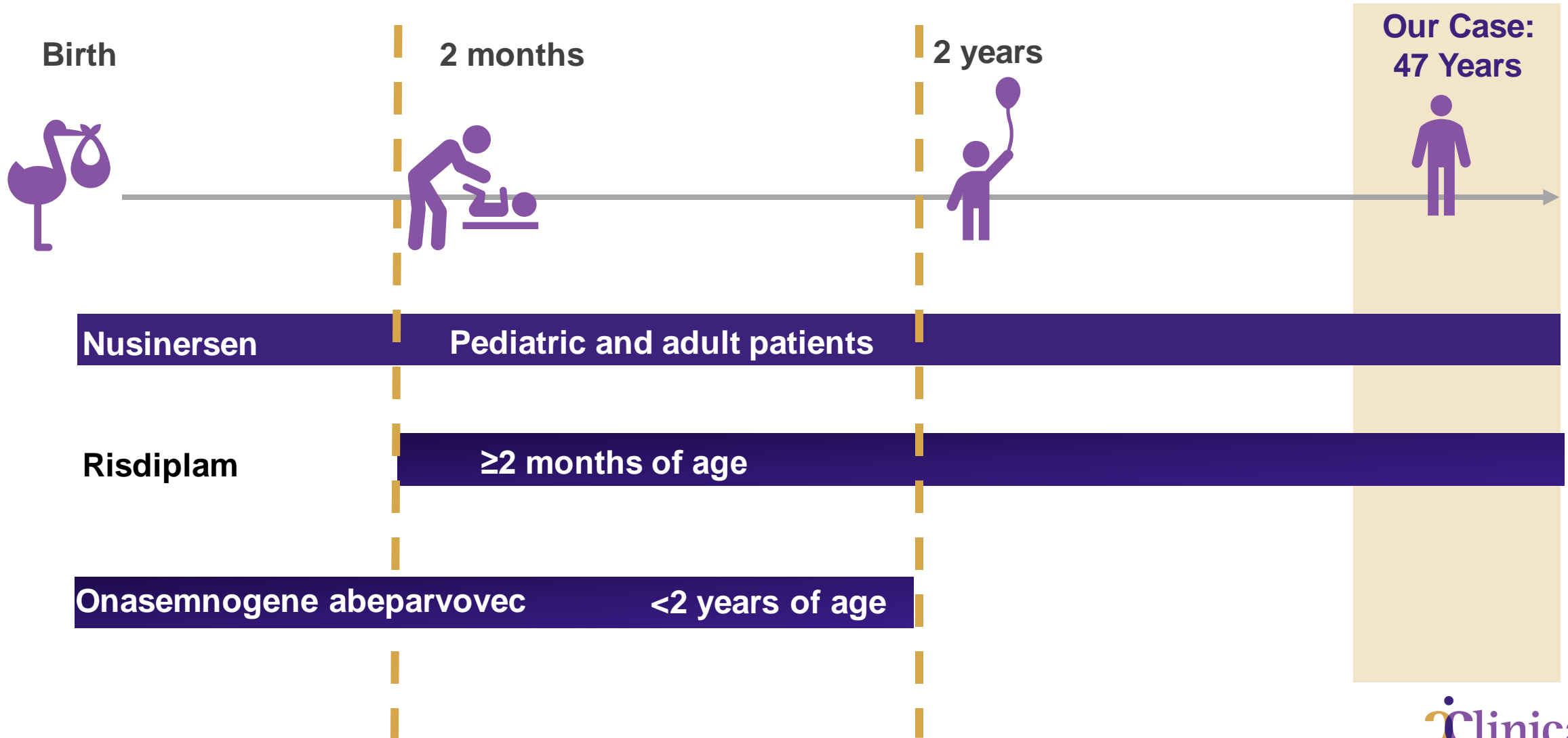
Functioning

He is walking now, but he may lose his ability to walk in the future due to the progressive nature of SMA

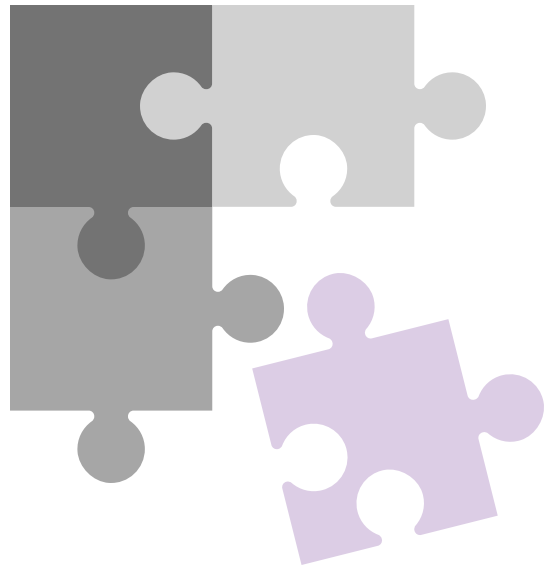
Treatment Selection

- Explain risks and benefits of each available option
- Discuss the logistics administering each agent

SMA Disease-Modifying Therapy



Nursing Considerations



Assessment

- What kind of loss of function is he having?
- Increased respiratory weakness?
- How is his nutrition and caloric intake?

Preserve Functioning

- May be prudent to start him on something
- Treatment can help preserve some of his functioning and maintain his QoL

Treatment Selection

- Understand where he is in life and select the treatment that can best be integrated into his lifestyle.

Fertility Considerations with Risdiplam



- Be respectful
- Do not make assumptions
- Bring up the topic with males and females
- Listen to their concerns
- Be inclusive in your approach
- Discuss fertility options



Thank you

*Please remember to complete the post-test and evaluation
so you can receive CE credit.*