Spinal Muscular Atrophy: Acute Management - Home and Inpatient

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• Disclosures:
  – Consultant, F. Hoffmann – La Roche, Inc. Research design and outcomes for intervention trial in children with SMA
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  – Consultant, Audentes Pharmaceuticals, Research design and outcomes for intervention trial in children with XL-Myotubular Myopathy
• Clinical Director, C.A.P.E. and Home Ventilation Program
• Member of Cure Spinal Muscular Atrophy, Medical Advisory Council
Objectives

• Outline “Hot Topics” in acute care management of individuals with SMA and their families

• Review limited published applicable research

• Posit questions and prioritize efforts to optimize subacute and acute care in the community and the hospital setting for individuals with SMA
Points of Reference


• ENMC Guidelines

• Evidence base

• Style and basic physiology
Not a red herring...but swimming upstream
General Considerations

• SMA Type (phenotype) – perhaps, changing with therapies
• Age and comorbidities
• Principles of subacute and acute care are extrapolated from guidelines for routine aerodigestive management
  – A. Adequacy of airway clearance
  – B. Ventilation and potential need for increased support
  – C. Potential for secondary complications or infections
  – D. Nutritional provision to minimize potential detrimental effect on function
• Anticipatory guidance is crucial for scheduled or unscheduled hospitalizations
• Family-centered
Anticipatory guidance / planning

• Discuss contingencies prior to acute events and as part of pre-procedural planning

• Set thresholds for families or older individuals with SMA with respect to signs and symptoms (or lack of traditional evidence of distress) as well as hypoxia and tachycardia as harbingers of significant illness

• Acknowledge parental/provider fatigue as a factor in determining threshold for presentation / admission

• Plan for care options when away from home

• Goals of care, advanced directives, healthcare proxy, MOLST/POLST
Engaging primary care and primary team

- Contact with the primary care team (generalist and specialty providers) is essential before, during, and at discharge. Reasoning is implicit.


Transport considerations and Emergency Department Evaluation

- Local emergency services (*first responders*) should be made aware of the child’s needs prior to an emergency.
- **Distance** from health care facility will also determine need for EMS
- **Degree of illness** of the individual (case-by-case) also dictates mode of transportation to health care facility
- **Family transportation** during subacute events, assuming that respiratory support and monitoring provisions versus EMS.
- For children with SMA I/II, emergent transportation should be provided via EMS with **ACLS capacity** and pediatric providers. If available a dedicated **pediatric transport team** would be recommended.
- The family should bring **home equipment** (e.g., **BiPAP with battery capacity**, cough assist, mask interfaces, gastrostomy adaptors,…) …
- **Brief summary** of medical needs, list of primary providers, and care protocols.
Emergency Department Evaluation continued…

• Small centers may not be familiar with SMA

• Large centers may not have protocols in their ED or are challenged to implement, too many rotators and so many staff.

• Contact primary neuromuscular teams for recommendations

• Which hospital?
Site of hospitalization (Community vs. Tertiary/Specialty Center)

• Presentation to the closest facility should be considered based upon the child’s degree of illness, distance from a tertiary facility, availability of pediatric transport team, environmental considerations, and goals of care.

• A high level of community hospital engagement if patient is > 30 miles (Golden hour?) from a pediatric or tertiary hospital.

• Children with SMA I or II should be hospitalized at a tertiary center, whether scheduled or emergent.

• Individuals with SMA III can consider hospitalization at a community hospital, but access to specialty consultation is recommended. Change in clinical status should prompt transfer to a center familiar with SMA.

• Provider/institutional variability regarding technology offerings.
Site of inpatient care
(i.e., general ward, intermediate or specialty unit, ICU)

- Triage depends upon **SMA Type**, clinical status, goals of care, and institutional specific resources. All providers, RNs, MDs, RTs, PAs,… should be aware of nuances of respiratory assessment and SMA management.

- Children with SMA Type I or II and acute issues should be cared for in intermediate care units or critical care setting.

- Children and young adults with SMA Type II without an acute respiratory diagnosis or minimal potential for secondary compromise can be considered for admission to a general ward, if routine respiratory support can be provided.

- **Early pulmonary/primary team evaluation** to assist in triage / direct admission

- Individuals with SMA Type III without acute respiratory diagnoses can be triaged to general wards.
Pre-procedural Screening / The planned admission

• Variable provider preferences
• Published evidence is limited but does not support routine cardiology evaluation unless there are other clinical indications.*
• Introduction and desensitization to non-invasive supports (cough assist and BiPAP) should be considered for scheduled procedures. The role of polysomnogram is debated.
• Nutritional assessment and periprocedural planning/optimization
• Low threshold to defer anesthesia and non-emergent procedures during intercurrent illness.
  – (Consider frequency of repeated procedures and time of year)
• Full disclosure of complexities of post-operative care with transitions from ETT mechanical ventilation to NIV.

Anesthesia / Procedural Sedation

- Anesthesia and procedural sedation should be provided at tertiary care centers familiar with SMA and respiratory support protocols.
- No such thing as “conscious” sedation
- Monitoring should include capnography as well as routine measures.
- Decision about use of NIV versus endotracheal intubation is reviewed with anesthesia, critical care, primary team, and the family/individual.
- Key issues - Difficult airway assessments, choice of agents, opiates, regional analgesia, extubation criteria
- ICU for post-procedure management, engaging primary respiratory providers.
- Don’t be afraid of opiates, as people in pain don’t breathe well either


Respiratory Protocols

• Consensus supporting the use of early and aggressive respiratory protocols that emphasize the use of preemptive measures, noninvasive supports, the use of positive pressure and augmented secretion clearance prior to empiric oxygen supplementation, clear extubation criteria, and attention to aerodigestive issues.

• Thresholds for intubation and goals of intubation should be established at the outset.

• Empiric antibiotics, viral testing, expectorated sputum for culture & sensitivity…?


Fasting / Nutrition Management / Dysmotility

- Avoid fasting

- Early and aggressive fluid and nutrition (either parenteral or enteral depending on frequency or airway treatments)

- Hold oral challenges for infants and those with possible oropharyngeal dysfunction, during acute illness, but consider alternatives for caloric provision.

- Use of prokinetic agents and opiate antagonists as well as routine bowel regimens
Comprehensive Acute Care

- Families are integral team members in care provision
- Skin care, PT/OT, Enhanced communication
- Engage primary providers


The ICU is not a vacation

1. Know my child’s baseline
2. Integrate and bridge multiple services
3. Disconnect between role of parent at home vs. parent in the PICU
4. PICU care does not equate with respite
5. High stakes learning environment
6. Heterogeneity within group
7. Lack of fit within the acute care model

Discharge Planning

- The child/young adult does not need to be back to baseline, depending upon comfort of the family and medical providers.

- Augment home support services

- Identify threshold for returning to the hospital as well as contingency planning

- Earliest discharge possible to minimize cumulative morbidities

- Contact primary teams (generalist and specialist) prior to discharge for follow-up planning.
Other considerations

• Consistency in homecare equipment provision / proactive not reactive

• Educating acute care providers
  – SMA Today vs. “Cult of Cure”
  – Neuromuscular respiratory care and acute respiratory distress – A Venn Diagram

• Pregnancy management

• Adult hospital transitions

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Optimizing Acute Care for SMA

- Acknowledge QoL
- Early discussions regarding interventions
- When considering technology support, present both benefits and potential difficulties, while also acknowledging that technology is not irrevocable
- Multidisciplinary approach
- Engage non-acute providers at critical times
- Acknowledge parent-child relationship and longitudinal perspective
- Extending critical care services beyond the boundaries of the ICU
- Transitions in Care
- Don’t Ignore faith
- Model effective and compassionate care with colleagues and trainees

Revisiting our Objectives

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