Background

- Gene replacement therapy (GRT; or "gene transfer therapy") is an emerging treatment strategy for some forms of neuromuscular disease.
- GRT differs from conventional treatments in several ways and requires in-depth infrastructure and coordination to ensure GRT is administered safely and effectively.
- This document captures highlights from an MDA webinar with care teams who have extensive GRT expertise. View the CE-accredited companion webinar here

Overview: Infrastructure & Coordination Considerations

Challenges of GRT implementation can occur (1) during start-up and administration and (2) later when caring for and ensuring adherence to follow-up recommendations

Hospital Administration	Neuromuscular Care Team	Finance	Pharmacy	Infusion Site and Staff
 Budget planning Staffing considerations Designated infusion space Prioritizing service line 	 Expertise Commitment/ establishment of specialized team Supporting education and participation with the team Complete all necessary tests/ procedures Post-infusion care Patient and family education 	 Explore eligibility, benefits, OOP costs Contracting (single case agreements) Revenue cycle management/ reimbursement tracking 	 P&T Ordering drug Storing drug Preparing/ administering product 	 Education Investment in outcomes Administering medication Ensuring safety day of infusion Monitor patient

Continued comprehensive care for rare disease

Content derived from Proud, C. MDA Clinical & Scientific Conference. 2023. Practical Considerations in Gene Therapy Session.

Consideration: How Will You Acquire and Deliver the GRT?

How a GRT product is procured is influenced by policies of the state, clinic, and insurers.

"Buy and Bill" Model:

- Hospital purchases drug, submits bill to insurance, then reimbursed
- Financial risk (or benefit) assumed by hospital

"White Bagging" Model:

- Hospital does not purchase; works with specialty pharmacy to acquire
- No financial risk assumed by hospital, but no reimbursement (storage, prep)

Options for acquisition impacted by:

- Local regulation
- Hospital policy Hospital risk
- tolerance
- Insurance policy

Resource: 340B

- A US Government program that requires drug manufacturers to provide outpatient drugs to eligible health care organizations at reduced prices.
- May help facilitate acquisition and impact on revenue for administering sites. More information here

Content derived from Proud, C. MDA Clinical & Scientific Conference. 2023. Practical Considerations in Gene Therapy Session.



Gene Replacement Therapy in Neuromuscular Disease: Practical Considerations & Tools (cont.)

Example Workflow: Patient Identification & Intake

Submit start/enrollment form

- Acknowledges intent to treat Enrolls patient in manufacturer
- "hub" services: • Benefit investigation
 - Assistance with the
 - appeal process, if needed
 - Copay assistance enrollment (when qualify)
 - Nurse education and
 - support

Set expectations with familie

- Benefit investigation and prior authorization with insurance
- Once authorization is obtained, involvement from contracting group
- Ordering and shipment of drugPre- and post-
- infusion requirements

Begin prior authorization process

- Submit all clinical documentation
- Depending on cost of therapy, hospital contracting group may need to obtain single case agreement to ensure appropriate reimbursement rate
- Determine if ordering through wholesale or specialty pharmacy

Example Workflow: Authorization & Approval

Setting up

- Having a designated rep, who is knowledgeable, about GT in regular (e.g. daily, weekly) discussions with clinicians and pharmacy team can help facilitate process.
- Identify the individual or group who facilitates GRT authorization
- Washington University in St. Louis example: email distribution list

Determine required data/documentation

- Auth request should include all requirements in the label (e.g., DNA/antibody testing, PT evals, clinic notes, etc.)
- Look for payer-specific policies (from website or manufacturer rep.) and "speak to the policy" in documentation (underline, bold, copy/paste policy)
- Consider submitting letter of medical necessity without a request

May need to wait for some testing to come back before starting the insurance process.

Example Workflow: Pre-Administration

Establishing care within NMD Center	Care coordination	Safety	Efficacy
 Baseline assessments with PT/OT Evaluate respiratory status – Pulmonology, RT Evaluate cardiac status Genetic counseling Supportive accommodations with Physiatry 	 Need for nursing/ social work/ care coordination/ access specialist role Families may need assistance with transportation, lodging for extended periods, financial assistance, etc. Set local protocols for patient management 	 Pursue baseline labs Ensure no underlying conditions that preclude safe therapy Ensure no recent or active illnesses around time of treatment Review recommendations for vaccinations Commitment by family regarding treatment journey/ adherence 	 Long-term follow-up assessments Referrals as appropriate to outpatient physical, occupational, speech therapies



Gene Replacement Therapy in Neuromuscular Disease: Practical Considerations & Tools (cont.)

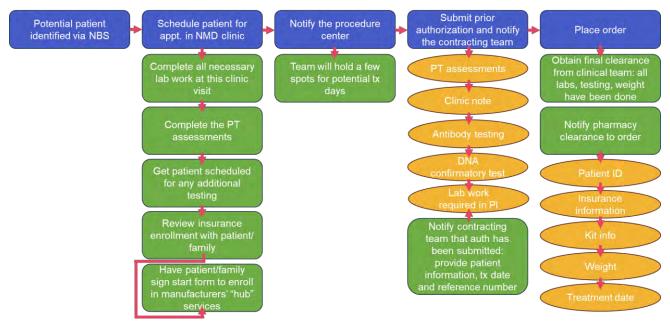


Considerations: Administration

Considerations for different types of infusion centers: General hospital center, Specialty center, In-patient unit in a non-in-patient status

- · Location: main hospital, hematology/oncology unit, general floor, PICU, neuroscience unit
- Impact of insurance authorization (inpatient versus outpatient)
- Volume of patients
- · Hours of operation to support infusion/observation
- Availability of scheduling (pre-planned vs. short notice)
- Staffing
- Pharmacy considerations: thaw and prep-time, etc
- Comfort level/training of staff in handling product
- Need for communication with registration/nursing for room placements
- Contingency planning for vascular access issues, infusion reactions, etc

Example Case & Workflow: SMA



Graphic courtesy of Collins, E.



Gene Replacement Therapy in Neuromuscular Disease: Practical Considerations & Tools (cont.) A Resource for Clinicians

Resources for Clinicians

Publications & Tutorials	MDA– Clinician Resources
 Petrich J. Gene Replacement Therapy: A Primer for the Health-system Pharmacist. <u>J Pharm Pract.</u> 	Gene Therapy Learning Modules —coming soon! MDA
2020;33(6):846-855. doi:10.1177/0897190019854962	Clinical Support
340B Educational Resources: www.hrsa.gov/opa/educational-resources	Sarepta GRT Enrollment form (DMD): <u>www.sarepta.com/sareptassist</u>
ASGCT website: <u>www.asgct.org</u> American Society of Gene + Cell Therapy	Novartis One Gene Program (SMA): <u>https://www.onasemnogene</u> <u>abeparvovec.com/onegene-program</u>

Resources to Share with Patients



Access companion CE-accredited MDA webinar here

