

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
<ul style="list-style-type: none">• Budget planning• Staffing considerations• Designated infusion space• Prioritizing service line	<ul style="list-style-type: none">• 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	<ul style="list-style-type: none">• Explore eligibility, benefits, OOP costs• Contracting (single case agreements)• Revenue cycle management/ reimbursement tracking	<ul style="list-style-type: none">• P&T• Ordering drug• Storing drug• Preparing/ administering product	<ul style="list-style-type: none">• 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:

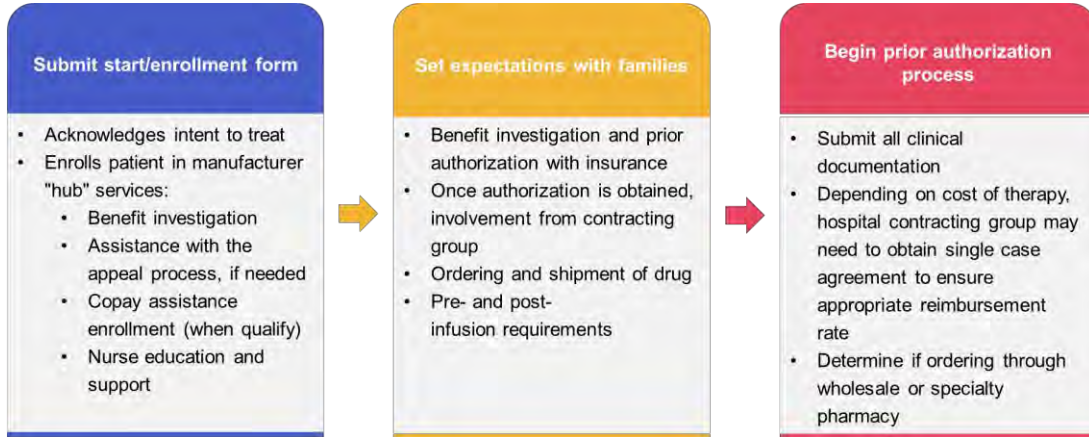
- Local regulation
- Hospital policy
- Hospital risk tolerance
- Insurance policy

Resource: 340B

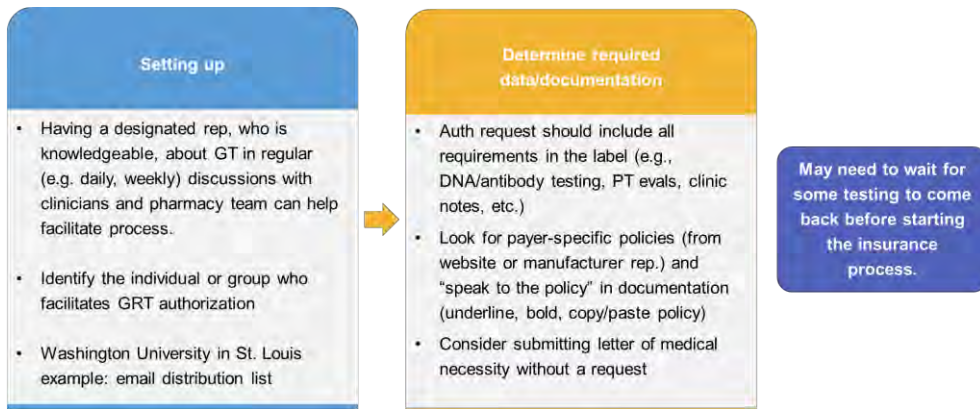
- A US Government program that requires drug manufacturers to provide outpatient drugs to eligible health care organizations at reduced prices.
- May help facilitate acquisition and impact on revenue for administering sites. [More information here](#)

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Example Workflow: Patient Identification & Intake



Example Workflow: Authorization & Approval



Example Workflow: Pre-Administration

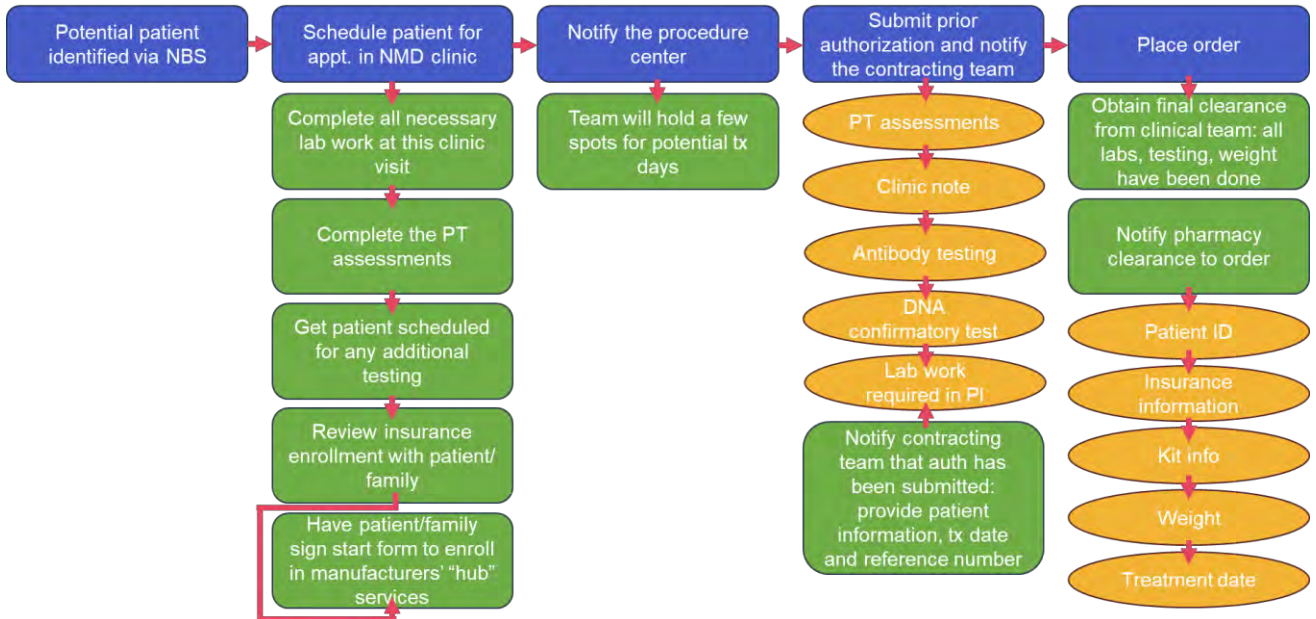


Considerations: Administration

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.

Resources for Clinicians

Publications & Tutorials

- Petrich J. Gene Replacement Therapy: A Primer for the Health-system Pharmacist. *J Pharm Pract.* 2020;33(6):846-855. doi:10.1177/0897190019854962

- **340B Educational Resources:** www.hrsa.gov/opa/educational-resources



- **ASGCT website:** www.asgct.org



MDA– Clinician Resources

Gene Therapy Learning Modules
—coming soon!



Clinical Support

- Sarepta GRT Enrollment form (DMD): www.sarepta.com/sareptassist
- Novartis One Gene Program (SMA): <https://www.onasemnogeneabeparvovec.com/onegene-program>

Resources to Share with Patients

MDA--Community Resources

www.mda.org/care/gene-therapy-community-support



- Virtual Learning: Gene Therapy 101 Webinar
- Virtual Patient Support Groups
- Print-Ready materials



ASGCT- Patient Education

www.asgct.org



Patient Access Programs

1. Find a disease fund - PAN Foundation
2. The Assistance Fund (tafcares.org)
3. Good Days | Effective Compassion | Copay Assistance (mygooddays.org)
4. National Organization for Rare Disorders | NORD (rarediseases.org)



Access companion CE-accredited MDA webinar [here](#)