**DMD Gene Therapy Clinician Instruments**

**RTI International and Duchenne UK**

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**Pre-Interview Clinician Online Survey**

You are eligible for this study if you are:

* A clinician with experience caring for patients with Duchenne muscular dystrophy.
* At least 18 years old.
* A resident of the United Kingdom or the United States.

You must be able to read and answer an online survey in English and later participate in an interview using Zoom.

* Your age:
  + [age drop down—under 18 leading to a “you do not qualify” message, by year 18–80, then ‘older than 80’]
* Your country of residence:
  + United Kingdom
  + United States
  + Other country (leading to a “you do not qualify” message)

**[----------------------------------------New screen----------------------------------------]**

**[CONSENT SCRIPT]**

**[----------------------------------------New screen----------------------------------------]**

* How do you describe yourself? (Mark one answer)
  + Male
  + Female
  + Trans Male/Trans Man
  + Trans Female/Trans Woman
  + Genderqueer/Gender Nonconforming
  + Different Identity
* What is your area of speciality? (text box)
* Number of years in specialty:
  + [Drop down 1 to 20, more than 20]
* What is the approximate number of patients with Duchenne muscular dystrophy that you see per year in your clinical practice? (text box)
* What is the approximate number of patients with Becker muscular dystrophy that you see per year in your clinical practice? (text box)
* Which best describes your patients?
  + Pediatric only (under 18)
  + Pediatric and adult (18+)
  + Adult only (18+)
* Which best describes your experience as part of a clinical trial team? (Choose all that apply.)
  + I have been part of a trial team for a DMD clinical trial that was **not** a gene therapy trial.
  + I have been part of a trial team for a DMD clinical trial for gene therapy.
  + I have been part of a clinical trial team for a condition other than DMD, that was not a gene therapy trial.
  + I have been part of a clinical trial team for a condition other than DMD, that was a gene therapy trial.
* Which best describes your understanding of gene therapy? (Mark one answer)
* Never heard the term
* Have heard it, but don’t understand
* Have some understanding
* Understand quite well
* Understand and could explain to others

**[----------------------------------------New screen----------------------------------------]**

**[Survey close]**

**Clinician interview guide**

*Gene therapy knowledge/initial attitudes*

1. How much would you say you know about gene therapy?
   * How would you define gene therapy to a patient or family? How might you describe the difference between gene therapy and gene editing?
   * How do you feel about gene therapy as a treatment for human diseases?
   * What are your biggest questions?
2. Do you have personal experience as part of the research team for gene therapy trials?
   * If yes, describe
   * If no, do you anticipate such experience in the future?

*Gene therapy perceptions*

Let’s think about gene therapy as a possible treatment for Duchenne. I want to hear your gut reaction now. Later we’ll discuss more specific questions.

1. How important do you find gene therapy as a therapeutic approach for Duchenne?
2. What types of benefits would you expect from DMD gene therapy?
   * What degree of benefit would you expect?
   * Do you have specific hopes regarding the benefits?
   * How long do you expect the benefits to last?
   * How much benefit do you think is needed for gene therapy to be worth approving?
   * What would successful treatment mean for your patients?
3. What types of risks would you expect from DMD gene therapy?
   * What degree of risks would you expect?
   * How concerned are you about the risks?
   * How much risk do you think is acceptable?
   * How have recent reports of gene therapy adverse events impacted your views of gene therapy?
4. What information would you want to see to encourage your enthusiasm regarding a clinical trial for gene therapy?
5. Do you think all, some, or few of your patients with Duchenne could benefit from use of gene therapy? Why?
6. Are there other challenges or downsides that you think will impact the use of gene therapy?
7. When do you think a gene therapy will be approved in DMD, if ever?
   * What do you think is the biggest barrier to an approved gene therapy for DMD?
8. Have you talked about gene therapy with patients or caregivers? Who brings it up? What do you discuss? What about gene editing?
9. Do you have (or do you expect you would have enough time to adequately cover gene therapy (trials) in these conversations?
10. What would be your decision-making factors if there are multiple approved gene therapies?

*Clinical trial preparation and implementation questions (those involved in gene therapy clinical trials)*

1. How do you go about recruiting patients for gene therapy trials?
2. What are the key factors that will make an institution an appealing gene transfer trial site?
3. What additional resources do sites need for gene therapy that they don’t have today?
4. What other challenges might sites experience as they begin new gene therapy trials?

*Clinical trial preparation and implementation questions (those not involved in gene therapy clinical trials)*

1. Have you referred patients to gene therapy clinical trials? How do you determine who to refer?
2. Would you be interested in being an investigator on a gene therapy clinical trial?
3. What additional resources would your site need to be a gene therapy site that aren’t available today?
4. What other challenges might your site experience if you began a new gene therapy trial?

*Final question*

1. Think to the future and imagine an approved gene therapy. What do you anticipate will be the biggest challenges, if any, of gene therapy as an approved treatment?