How to advocate for your child with DMD

“Learning the lingo...”

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- Dad of Mikey and Reid, both living with Duchenne
- Clinical Trials Liaison: Nemours Alfred I. duPont Hospital for Children, Wilmington, DE for Dr. Mena Scavina (PPMD Certified Clinic)
So, how do I advocate?

Things I’ve learned over the years...
Different ways to advocate...

1. Get the name Duchenne Muscular Dystrophy out there.
2. Speak to local, state and national legislators about DMD
3. Teach family and friends the ins and outs of DMD
4. Educate the Educators
5. Look to enroll in a clinical trial
Where to start?

• Duchenne Registry!!! (Previously Duchenne Connect)
• For Duchenne or Becker muscular dystrophy or you are a female carrier of Duchenne or Becker.
• Your anonymous Registry data is shared with researchers to speed the development of new therapies.
Duchenne Registry

• 10-year-old network of patient-powered data that will be used to improve care for people living with Duchenne and increase our understanding of the disorder.

• Once you register and complete your Medical Surveys, we will let you know when you might be a good fit for research studies and clinical trials.

• Your data also helps drug developers know the size of the Duchenne population available for trials and helps identify new trial sites, increasing our community’s access to trials and potential therapies.
Next step....clinicaltrials.gov
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<table>
<thead>
<tr>
<th>Status</th>
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<th>All studies</th>
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<tr>
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### Understanding the lingo...

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<tr>
<th>Row</th>
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<th>Conditions</th>
<th>Interventions</th>
<th>Locations</th>
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| 3   |       | Completed | **Safety and Dose Finding Study of NS-065/NCNP-01 in Boys With Duchenne Muscular Dystrophy (DMD)** | • Duchenne Muscular Dystrophy     | • Drug: NS-065/NCNP-01          | • UC Davis  
  Sacramento, California, **United States** |
|     |       |           |                                                                            | • Drug: Placebo                   |                                 | • University of Florida Health  
  Gainesville, Florida, **United States** |
|     |       |           |                                                                            |                                   |                                 | • Lurie Children’s Hospital  
  Chicago, Illinois, **United States** |
|     |       |           |                                                                            |                                   |                                 | (and 4 more...)                                |
The safety and scientific validity of this study is the responsibility of the study sponsor and investigators. Listing a study does not mean it has been evaluated by the U.S. Federal Government. Read our disclaimer for details.

Sponsor:
NS Pharma, Inc.

Collaborators:
Nippon Shinyaku Co., Ltd.
Cooperative International Neuromuscular Research Group
Therapeutic Research in Neuromuscular Disorders Solutions

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Information provided by (Responsible Party):
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ClinicalTrials.gov Identifier: NCT02740972

Recruitment Status: Completed
First Posted: April 15, 2016
Last Update Posted: July 29, 2019
Advocate early and often!!!
Ages Eligible for Study: 4 Years to 9 Years (Child)
Sexes Eligible for Study: Male
Accepts Healthy Volunteers: No

Criteria

Inclusion Criteria:
- Male ≥ 4 years and <10 years of age
- Confirmed DMD mutation(s) in the dystrophin gene that is amenable to skipping of exon 53 to restore the dystrophin mRNA reading frame;
- Able to walk independently without assistive devices;
- Ability to complete the time to stand, time to run/walk and time to climb assessments;
- Stable dose of glucocorticoid for at least 3 months

Exclusion Criteria:
- Acute illness within 4 weeks prior to the first dose of study medication;
- Evidence of symptomatic cardiomyopathy. [Note: Asymptomatic cardiac abnormality on investigation would not be exclusionary];
- Severe allergy or hypersensitivity to medications;
- Severe behavioral or cognitive problems that preclude participation in the study, in the opinion of the Investigator;
- Previous or ongoing medical condition, medical history, physical findings or laboratory abnormalities that could affect safety, make it unlikely that treatment and follow-up will be correctly completed or impair the assessment of study results, in the opinion of the Investigator;
- Patient is taking any other investigational drug currently or within 3 months prior to the start of study treatment; or
- Patient has had surgery within the 3 months prior to the first anticipated administration of study medication or surgery is planned for anytime during the duration of the study;
- Patient has previously participated in this study or any other study during which NS-065/NCNP-01 was administered.
What I did early and often

• Contact the **study collaborators** to find out any and all information/details

• Ask them for their name and number/extension

• Don’t be afraid to ask the 5 W’s and 1 H

• Give them all your information

• Ask if there is anyone else that you should talk to in their company/site

• Be ready to write everything down and try to keep detailed records
I contacted the TRINDS group and they gave me ALL the information that I needed!
What I did next:

- Looked up each locations website and found out who their Neuromuscular team was....and called them!
- Each site was very helpful and gave me as much information as they could.
- Ask for names, phone numbers and even email address.
- Be THAT parent!!!
Don’t be shy
Our experience for 121 weeks

• A day in the life of traveling
• Pick up rental car the day before
• Wake up at 3:30 am (Can get a hotel)
• Leave the house by 3:45 am
• Arrive in Richmond at 7:15 to 7:30 am
• Infusion by 9:30 to 10:00 am
• Leave by 12:30 to 1pm
• Arrive home by 5:30 to 6pm
How can we do this...

• Clinical trials pay for a plane, train or car, hotel, food, etc...
  • Different for each trial
• Grandparents or other family members are allowed to chaperone
FMLA...

• FMLA: Gives eligible employees the right to take up to 12 weeks of unpaid leave each year
  • To qualify for benefits, an employee must be employed with the company for at least 12 months and worked for at least 1,250 hours during the 12 months prior to the leave.
  • You can take the time all at once or in increments.
• Contact your HR person if possible and/or look up the details on [https://www.dol.gov/whd/fmla/](https://www.dol.gov/whd/fmla/)
Conclusion....

• You’ll never get answers to questions you don’t ask!
• Contact the Sponsor and the Collaborators
• Contact the sites
• Talk to your employer
• Advocate for your sons!
Thank you!