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The Value of Gene Therapy in Duchenne

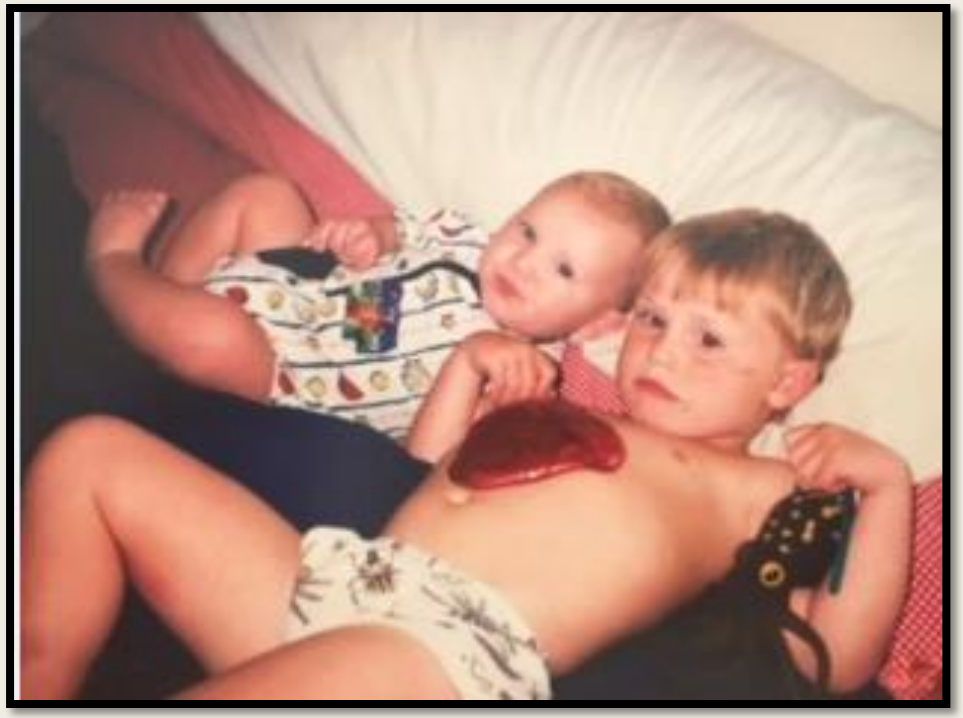
Jenn McNary
Patient Advocate, Consultant, and Caregiver

February, 2002.
Diagnosis: Duchenne MD, del exon 52

"Take them home and love them, there is nothing you can do, they will steadily decline, need a wheelchair and eventually die of heart or respiratory failure."

Early childhood symptoms

- Gross motor delays
- Toe walking
- Frequent falls
- Gower maneuver
- Fatigue



Impact

The whole family is impacted by a duchenne diagnosis

- Emotional-, grief, guilt, depression, isolation
- Financial- Cost of care, medical interventions, equipment, home modifications, loss of wages for caregiver
- Physical- Falls, osteoporosis, bone breaks, fatigue, frequent infections, side effects of steroid treatment, heart failure, respiratory infections
- Educational- Learning disabilities, cognitive delays, increased autism diagnosis, missing school days

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Meaningful Endpoints

What matters to patients and caregivers?

- Increased independence
- Greater quality of life
- Slowed or halted progression
- More energy
- More mobility
- Better ability to breathe

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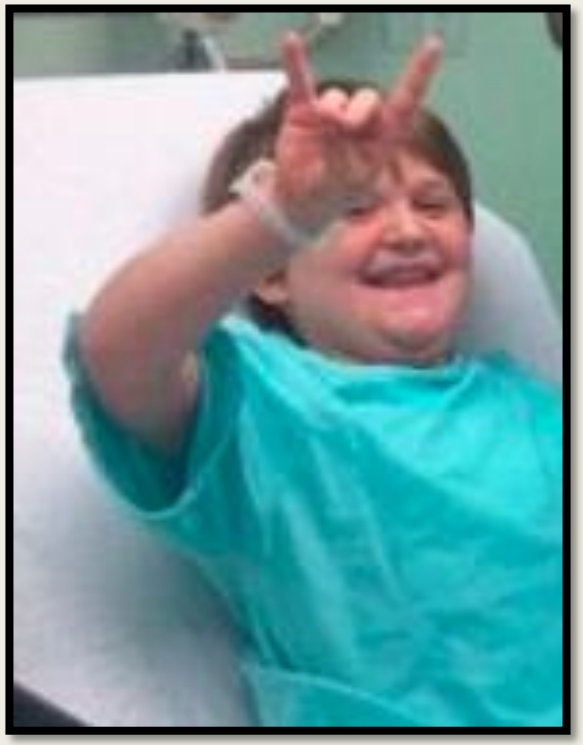
August, 2011
Eteplirsen Clinical Trial Enrolls Max

6 months into the study, Max's disease trajectory changed

- At 9.5 he stopped falling
- He was able to walk most of the day, after using a wheelchair previously
- He opened drink containers
- Less Fatigue



Current Regulatory Landscape

FDA recognizes the urgency to move new safe and effective therapies to the clinic faster

- Fast Track-Fast track is a process designed to facilitate the development, and expedite the review of drugs to treat serious conditions and fill an unmet medical need
- Breakthrough-A process designed to expedite the development and review of drugs which may demonstrate substantial improvement over available therapy
- Accelerated Approval- These regulations allowed drugs for serious conditions that filled an unmet medical need to be approved based on a surrogate endpoint
- Priority Review- A Priority Review designation means FDA's goal is to take action on an application within 6 months

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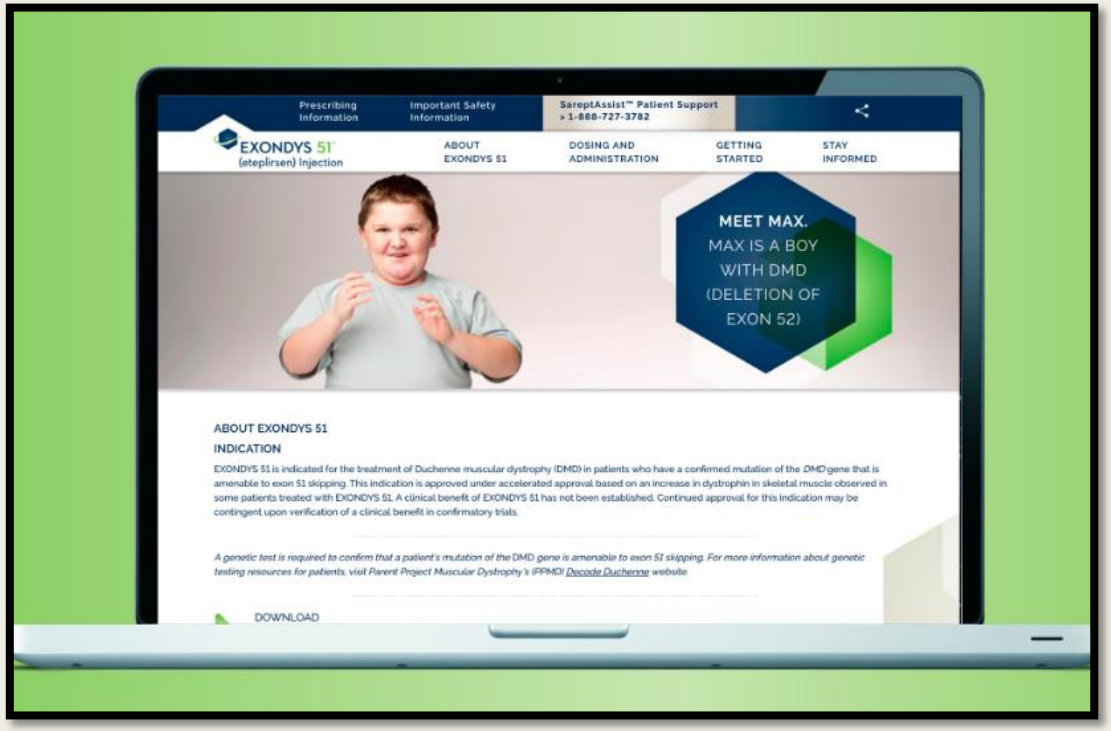
2016, Exondys51 Approval

First disease modifying duchenne approval

- Slows the progression
- Extends Walking Years
- Reduces Fatigue
- Stabilizes Pulmonary and Respiratory Function
- Stepping stone to Further Innovation for Duchenne



Progress

- Steroids + 2 years delayed loss of ambulation = 5-7 Years added life span
- Exondys51 + 3 years delayed loss of ambulation added to steroids
- Gene Therapy ?? Potential to be transformative

Current Reimbursement Landscape:

Approval does not equal access

- Faster approvals utilizing more flexible pathways is often seen by payers as a reason to deny coverage
- Entities like ICER are de-valuing rare disease patients lives, and giving payers further permission not to cover these therapies
- Patients' families are fighting for access to equipment, care, and treatment.

The New York Times

Insurers Battle Families Over Costly Drug for Fatal Disease

The boys' insurer, Excellus BlueCross BlueShield, refused to cover the cost of the drug because the twins, who are 15, can no longer walk. Their disease, Duchenne muscular dystrophy, overwhelmingly affects boys and causes muscles to deteriorate, killing many of them by the end of their 20s.

"I'm cycling between rage and just sadness," their mother, Alison Willis Hoke, said recently, on the day she learned that an appeal for coverage had been denied. For now, the company that sells the drug, Sarepta Therapeutics, is covering the treatment's costs, but Mrs. Hoke does not know how long that will last.

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Looking forward – Pipeline:

Duchenne has Many Potential Therapies in Clinical Trial

- Restore or Replace Dystrophin
- Fibrosis
- Inflammation
- Calcium
- Muscle Growth & Protection
- Restoring Cell Energy
- Improving Heart Function

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Hope for the future

Gene Therapy offers patients hope for a one-time curative treatment

- Early Diagnosis via Newborn Screening
- Early Intervention
- Disease modifying
- Restoration of Dystrophin
- Prevention of Muscle Breakdown
- A Future Without Duchenne

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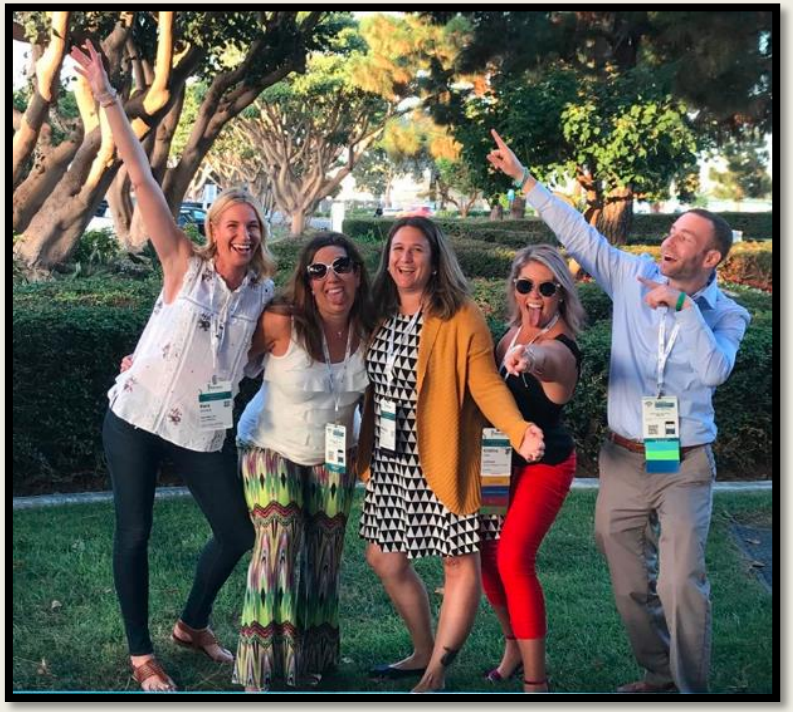
Advocates Needed

- Flexible trial designs- Advisory Boards, PFDD meetings
- Accelerated approvals- Adcoms, PROs
- Value Assessments/ ICER – Educate yourselves and others, Voice concern, make noise
- Access and Reimbursement – Attend DUR and P&T committee meetings,
- Oversight- Hill Days,



#NotWorthless

A watchdog group sets aside emotions to assess drugs' value. Patients say their lives are more than a number

By KATE SHERIDAN @sheridan_kate / AUGUST 2, 2019

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Thank you!

Keep in touch

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