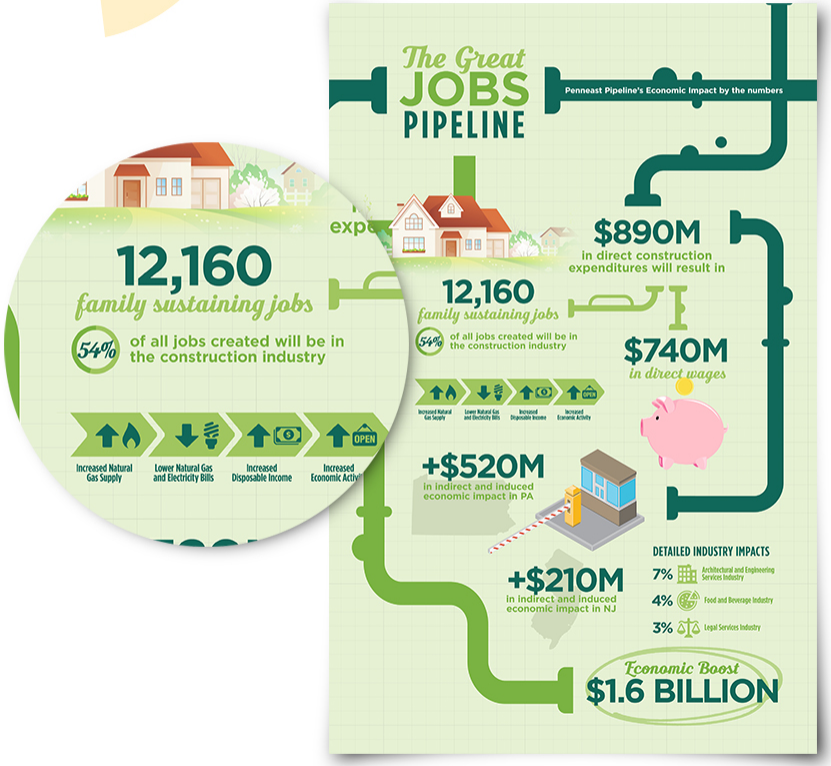
INFOGRAPHIC



"Clearly it's a breakthrough drug, it has minimal side effects. I say minimal, we haven't seen a single one, which is incredibly remarkable, and we unequivocally showed efficacy over a two year period which is unheard of in clinical trials. If you add up all these things, that is the ticket for accelerated approval by the FDA, and we need help getting there." - *Dr. Jerry Mendell*

DUCHENNE MUSCULAR DYSTROPHY (DMD) A child of five diagnosed with Duchenne experiences a loss of muscle strength leading to wheelchair confinement by adolescence and a shortened life span assisted by ventilators. The progressive deterioration of muscle strength leads to death in the late teens or early twenties.

UNIVERSALLY FATAL CHILDHOOD DISEASE

#1 GENETIC KILLER OF CHILDREN

1 IN 3,500 MALE BIRTHS

350,000 CHILDREN WORLDWIDE

24,000 BOYS IN THE US

NO APPROVED TREATMENT

"They are stabilized. They are making dystrophin. This is really quite an amazing feat. I support this." - *Dr. Louis Kunkel, Harvard Medical School*

THE RACE TO YES MILE MARKERS

1986 | The genetic defect that causes Duchenne is discovered, jumpstarting a decades-long marathon by scientists and parents to find a cure.

2009 | Leading scientists believe a key clinical study on "Drisapersen" will prove the efficacy of a DMD breakthrough therapy. The development spurs renewed urgency to discover a cure.

JULY 2012 | Headlines herald a major clinical breakthrough for DMD research with evidence that after 36 weeks of treatment, eteplirsen delays the progression of the deadly disease. Today, scientists have 120 weeks of successful clinical data. The "Race to Yes" begins!

JULY 2013 | Based on positive clinical outcomes, the FDA expresses willingness to consider a New Drug Application (NDA) for eteplirsen.

FEBRUARY 2014 | Seven of the world's leading Duchenne scientists and clinicians brief FDA leadership and 100 congressional staffers on the success of eteplirsen clinical trials, endorsing approval for the therapy. The effort is publicly backed by a bipartisan coalition of members of Congress, resulting in an urgent letter to the FDA.

MARCH, 2014 | The Race to Yes launched and gathered over 100,000 signatures on a White House Petition urging the FDA to Say Yes to Accelerated Approval for safe, effective therapies for children with Duchenne.

EARLY 1990'S | The FDA creates the Accelerated Approval pathway to expedite therapies in response to the HIV/AIDS epidemic. (To receive AA, a drug must address a life-threatening condition and demonstrate effects reasonably likely to predict a long-term, clinical benefit.)

2012 | President Obama signs the bipartisan Food and Drug Administration Safety and Innovation Act (FDASIA) to expedite "development and review of innovative new medicines...including those for rare diseases" using the Accelerated Approval pathway.

2013 | DMD parent advocates hold the first in a series of discussions with the FDA to urge use of the Accelerated Approval pathway for eteplirsen.

NOVEMBER 2013 | The FDA rejects Sarepta, calling the NDA for eteplirsen "premature," despite ongoing strong, positive outcomes in clinical trial.

FEBRUARY 2014 | Thousands of parent advocates launch "The Race to Yes" campaign during International Rare Disease Week. The nationwide effort urges the FDA to say Yes to Accelerated Approval pathway for safe, effective DMD therapies.

APRIL, 2014 | Sarepta Therapeutics sold it received guidance from the Food and Drug Administration that will enable it to apply for accelerated regulatory approval of eteplirsen by the end of this year, before completing a larger scale, confirmatory clinical study.

THE ROAD TO YES

URGENT NEED

Approving safe, effective and proven therapies for Duchenne Muscular Dystrophy — the leading genetic killer of children in the United States.

SOUND SCIENCE

In clinical trials the eteplirsen treatment has successfully arrested the disease's progression. The drug has been well tolerated, with no clinical treatment-related adverse events, no serious adverse events, no discontinuations and ongoing stabilization.

RIGHT POLICY

Pursuing the same accelerated drug approval pathway provided to HIV/AIDS and cancer for urgent, deadly diseases with no treatment.

SAY YES!

#RACETOYES

THE GOAL IS TODAY | Children with Duchenne score huge when the FDA says "YES" to Accelerated Approval for eteplirsen. A YES for this first safe and effective Duchenne therapy will pave the way for additional treatments to cross the same finish line in time to benefit this generation of children with Duchenne.

RUNNING THE NEXT LAP IN
THE RACE TO YES
SAFE + EFFECTIVE = YES!

LAP 1

PARENTS LAUNCH "THE RACE TO YES," INITIATE WHITE HOUSE PETITION *(FEBRUARY 2014)*

- Urge FDA to reverse course and embrace faster, more efficient drug development.
- Scientist-backed approach, strong bipartisan coalition in Congress.
- Race to Yes White House petition reaches the 100,000 signatures required for response.

LAP 2

FDA ADOPTS RACE TO YES RECOMMENDATIONS *(APRIL 2014)*

- FDA reverses course, lays out path forward for Duchenne therapies, including eteplirsen.
- FDA provides guidance for eteplirsen manufacturer for additional testing required.
- White House still silent on petition, now topping 106,000 signatures.

LAP 3

TIME=MUSCLE, DRUG MANUFACTURERS, FDA SET THE PACE & SCOPE FOR ACCESS TO TREATMENTS. *(HAPPENING NOW)*

- Every child with Duchenne must be given access to safe, effective treatments.
- Children must be spared from unnecessary placebo studies, insuring that good science serves children.
- President Obama must support this accelerated approach to treatment.
- FDA, drug manufacturers must have more frequent, in-depth collaboration.

FOR EVERY CHILD WITH DUCHENNE, *TIME IS MUSCLE.*
ACT NOW. JOIN THERACETOYES.ORG