

THE ORPHAN DRUG ACT: SPURRING INNOVATIVE RARE DISEASE DRUG DEVELOPMENT

The **Orphan Drug Act** was enacted in 1983 to incentivize innovative drug development for the nearly **7,000 identified rare diseases**. Prior to 1983, only a small number of therapies were approved to specifically treat rare diseases, leaving millions of patients within the United States and the rest of the world without treatment.

Incentives provided by the Orphan Drug Act have encouraged a greater number of companies to develop therapies for rare diseases. Many are small companies that do not yet have products on the market and do not generate revenue.

Prior to the enactment of the Orphan Drug Act in 1983, very few companies developed therapies for rare diseases. Today, more than **595** companies are developing therapies in the rare disease space.



Incentives provided by the Orphan Drug Act have dramatically changed the number and types of therapies that are available to patients with rare diseases.

In the last 5 years alone, **21 novel first in class treatments** were approved for treating rare diseases, including:

Duchenne Muscular Dystrophy

MPSVII (Mucopolysaccharidosis type 7)

Spinal Muscular Atrophy

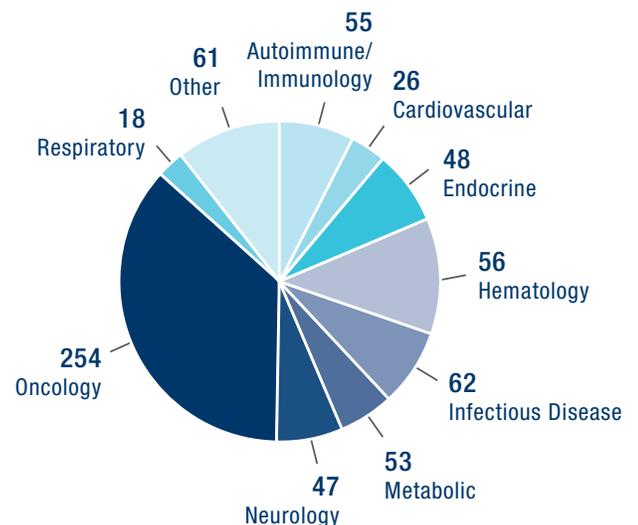
X-linked Hypophosphatemia

Source: www.accessdata.fda.gov | FDA Orphan Drug Designations & Approval
Biomedtracker | <http://www.biomedtracker.com>



▶ Prior to 1983, only a small number of rare diseases had approved therapies. Today, **539** rare diseases have approved therapies

TREATMENTS AFTER 1983

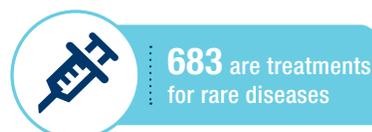


Source: www.accessdata.fda.gov | FDA Orphan Drug Designations & Approval

While progress has been made, there is still much work to be done.



Of the **695**...



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Rare Disease Patient Experiences with Orphan Drugs

CONNER BEISH

Conner was born healthy and developed normally until the age of 2, when his speech was delayed and he began speech therapy. After a series of seizures, which began around the age of 3, and several diagnoses, doctors felt genetic testing was necessary, after which he was finally diagnosed with a rare condition known as Batten Disease in January 2017.



At the time of diagnosis, there was only one trial available, which he did not qualify for because his disease was too far progressed. Fortunately, 3 months later, the therapy received FDA approval for CLN2 Batten Disease, and he began receiving treatments in June 2017. Since treatments began, Conner's quality of life has greatly improved.

We hope a cure is on the horizon but know it may not be possible in Conner's lifetime. The infusions, however, have extended his life, giving us many more years to make amazing memories with him!

ALEX REZKALLA

In the summer of 2015, our 4-year-old son, Alex, was diagnosed with Duchenne Muscular Dystrophy (DMD). His future went from being full of endless possibilities to having the likelihood of being dramatically shortened and ultimately out of our control.



Thanks to the pathway that the Orphan Drug Act created for rare disease drugs, our son's outlook was different. Since 2016, Alex has been a participant in a trial for a potential new orphan drug. As a result, Alex takes part in normal activities and entertains the nursing staff by dancing and running up and down the hallway or telling some of his best jokes.

While Alex's treatment is not a cure, it has improved his quality of life and offers us added time for him to be in our lives. This DMD mom is waiting and advocating for drug approvals and access to treatments for rare diseases so they can offer other families like ours hope and optimism.

RACHEL JONES

My mother was the first in her family diagnosed with X-linked hypophosphatemia (XLH). At the time, little was known about XLH, or how to treat it, which is why I was fortunate to receive a diagnosis when I was 6 months old. Throughout my childhood, it was difficult to find any doctors with knowledge of XLH, and, as a young adult, I stopped treatment, believing little was and ever would be known about the disease.



However, once I became a mother, and my children were diagnosed with XLH, my perspective changed. I found The XLH Network, which was a source of information for me and a place to connect with patients like me. Eventually, my desire to educate myself and advocate on behalf of my children grew into a desire to advocate on behalf of others with XLH. Current research and a promising new treatment give me hope for the future.

BAILEY WHITE

My first daughter, Madison, passed away at 7 months from SMA type 1. That's why we had our second daughter, Bailey, tested for SMA in utero where she was diagnosed by an amniocentesis. Early diagnosis enabled Bailey, when she was 3 months old, to enroll in a clinical trial assessing the safety and efficacy of a potential new orphan drug that targets the cause of SMA, and her quality of life has drastically improved as a result.



For Bailey, her orphan drug has been an almost perfect treatment. The breakthrough therapy has enabled her to take time out from her BiPAP machine, which helps her breathing – which is quite a relief – and tolerate being upright long enough to get some bear hugs from Mom or Dad.

Early detection and diagnosis not only improves outcomes and quality of life, it saves lives!