

Medical Research: It's About Max



“Every 3 weeks I get really angry and groggy and tired, and I get sick. And it happens every 3 weeks.”

When he was just 22-months-old, Max Hasenauer was diagnosed with X-linked agammaglobulinemia (XLA), a very rare genetic condition where the body does not produce the antibodies necessary to maintain a functioning immune system. In order to survive, he takes antibiotics daily and receives infusions of new antibodies every three weeks. In the days leading up to his infusion, he feels fatigued and ill as the old antibodies wear off. Despite his treatments, XLA can quickly become life-threatening. Max has even had to miss school during flu season to avoid contact with very ill people, who could jeopardize his health.

While new treatments are certainly needed to improve Max's quality of life, he would not be alive today without the research that made his infusions possible. The National Institutes of Health (NIH) has also launched the "Therapeutics for Rare and Neglected Diseases program," a collaboration between NIH researchers, nonprofit organizations and pharmaceutical and biotechnology companies to accelerate the development of new treatments for many rare diseases.



VON HIPPEL LINDAU SYNDROME
FAHRS DISEASE
MARFAN SYNDROME
XLINKED AGAMMAGLOBULINEMIA
TAY SACHS DISEASE
HUTCHINSON GILFORD PROGERIA
SICKLE CELL ANEMIA
CROHNS DISEASE
CREUZFELDT JAKOB DISEASE
NARCOLEPSY
MICROCEPHALY
DERCUMS DISEASE
FRAGILE X SYNDROME
HUTCHINSON GILFORD PROGERIA
WERNER SYNDROME
CYSTIC FIBROSIS
SICKLE CELL ANEMIA
LUPUS
ANGELMAN SYNDROME
MARFAN SYNDROME
EPIDERMOLYSIS BULLOSA
NEUROMYELITIS OPTICA
ALPHA 1 ANTITRYPSIN DEFICIENCY
FAHRS DISEASE
ANGELMAN SYNDROME
FIELDS DISEASE
DERCUMS DISEASE
TARDIVE DYSKINESIA
TARDIVE DYSKINESIA
NEUROMYELITIS OPTICA
WERNER SYNDROME
XLINKED AGAMMAGLOBULINEMIA
SPINAL MUSCULAR ATROPHY
VON HIPPEL LINDAU SYNDROM
LANDAU KLEFFNER SYNDROME
ALPHA 1 ANTITRYPSIN DEFICIENCY
FAHRS DISEASE
ANGELMAN SYNDROME
FIELDS DISEASE
DERCUMS DISEASE
TARDIVE DYSKINESIA
TARDIVE DYSKINESIA
NEUROMYELITIS OPTICA
WERNER SYNDROME
XLINKED AGAMMAGLOBULINEMIA
SPINAL MUSCULAR ATROPHY
VON HIPPEL LINDAU SYNDROM
LANDAU KLEFFNER SYNDROME

Approximately 7,000 different rare diseases affect 30 million Americans. From 1983-2013, over 2,900 potential rare disease treatments entered the research pipeline.^[4]

We've made progress. But the funding to sustain it is eroding.

Congress: harness your compassion, gather your will, and fund the National Institutes of Health at \$32 billion in FY15.

Do it for Max.

www.researchamerica.org
[1] www.rarediseases.org