
THE ZEBRA & THE BEAR
In the world of rare disease, nothing is more powerful than a mother's love.
A DOCUMENTARY FILM BY PATRICK O'CONNOR

SCENIC FILMS PRESENTS

THE ZEBRA & THE BEAR

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nothing is more powerful than a mother's love.

A FILM BY PATRICK O'CONNOR

Produced by Patrick O'Connor, Margaret McMullan and Amber Olsen

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2024 / U.S. / 84 minutes / English (Spanish subtitles available)

www.thezebraandthebear.com

Impact and Educational Distribution

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Click here to watch the official trailer

SYNOPSIS

THE ZEBRA AND THE BEAR is an intimate and impactful documentary film about how a mother's fierce determination to save her daughter from a fatal, ultra-rare genetic disease called Multiple Sulfatase Deficiency (MSD) leads her on a journey to raise millions of dollars and drive the development of a pioneering gene-therapy treatment.

Filmed over seven years, THE ZEBRA AND THE BEAR reveals the human story behind the science that may eventually save the lives of generations of children born with rare genetic disorders.

AUDIENCE REACTIONS

"Director Patrick O'Connor has captured the diagnostic and treatment odyssey faced by patients with rare diseases. This is a must-see film for all parents and healthcare providers."
– **Karen Summar, MD,** MS Developmental Pediatrician

"...Beautiful, important. Could not look away, except to find tissues."
– **Laura,** Rare Disease Parent

"A phenomenal film." – **Natalie,** Genetic Counselor

"... absolutely raw and heartfelt and very, very honest."
– **Alyssa,** Rare Disease Parent

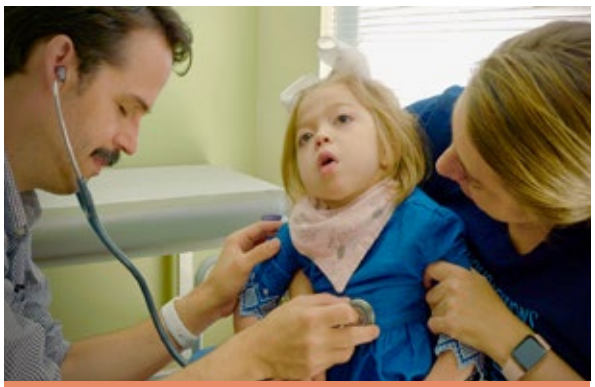
"It was so moving and real. I will feel the impact forever."
- **Leslie,** Rare Disease Parent

"Willow's mother takes us through the unimaginable trials required to improve her daughter's chances of survival, and in so doing, creates a path for many other families with rare disorders to follow."
- **Hans C. Andersson, MD,** Director, Hayward Genetics Center, Tulane University Medical Center

Dr. Steven Gray - Director of University of Texas Southwest Viral Vector Facility

Dr. Cathleen Lutz - VP Rare Disease Translational Center The Jackson Laboratory

Amber Olsen, the mother of a child with MSD at The Jackson Laboratory



Dr. Brian Kirmse - Pediatric Geneticist

DIRECTOR'S STATEMENT

I was introduced to Amber Olsen, the "mama bear" in the film, back in the spring of 2016 when a mutual friend asked if I would be willing to meet with the mother of a child with a rare disease who was looking for someone to make a short fundraising video. I had recently finished a documentary film called **THE INVISIBLE PATIENTS** about a nurse practitioner who cares for very ill homebound patients.

When we met, Amber told me the story of how her 2-year old daughter Willow had been diagnosed with a fatal, ultra-rare disease called Multiple Sulfatase Deficiency (MSD), an inherited genetic condition that leaves the body without the enzymes necessary to filter out natural waste by-product created by normal cellular activity.

At the time, there was no known treatment or cure for MSD, and the progression of the disease would be relentless and painful. As the cellular waste built up, all of Willow's vital systems – brain, muscles, vision, respiratory, digestive - would soon begin to fail. The doctor told Amber and her husband Tom to go home, treat her symptoms, and keep her comfortable until she died, likely before her 10th birthday. It was a devastating blow that would change the course of their lives in unimaginable ways.

Unwilling to accept that nothing could be done to cure Willow, Amber and her husband immediately went to work learning as much as they could about MSD —the science behind it, and any possible way forward that would help their daughter live.

Early on, Amber found a mother in Arizona whose child had a similarly deadly lysosomal disease. She told her that she had recently spent over $30,000 to purchase a herd of sheep in New Zealand, which she was planning to use for large animal testing. Amber's first thought was, "Am I supposed to buy my own sheep?"

As Amber told me this detail, I realized that there was a larger story here about an "ordinary" family embarking on a solitary quest to raise millions of dollars and push for a pioneering gene therapy treatment save their daughter's life. They had no road map, no guide, and the odds were stacked high against them. They were utterly unprepared, and as much as their friends and acquaintances sympathized with their situation, no one could truly understand what they were going through.

I thought theirs would be a fascinating story, but more importantly, I was struck by how unfair and, I'll say, immoral it was that in the year 2016, in the wealthiest country in the world, a mom and dad had to figure this out on their own.

Amber and her husband Tom agreed to allow me to make a film, and for the next seven years or so, I spent time with their family, went with them to fundraising events, Willow's appointments, meetings with scientists and specialists, and generally tried to capture a sense of their life as parents of a child with a fatal, ultra-rare genetic disease with no known treatment or cure.

Willow turned 11 in August of 2024 (her little birthday celebration was the last thing we shot), and the research that Amber pushed so hard for ultimately led to MSD being selected for a kind of pilot gene therapy research program at the NIH which all but guarantees that children with MSD will begin being treated in clinical trial within the next year or so.

The NIH Bespoke grant announcement is a real victory that will potentially lead to a gene therapy treatment for this terrible disease. Sadly, Willow passed away a few months after she turned 11 surrounded by her family and long-time caregivers. As her mother Amber said, "She's been freed from her MSD body."

Willow's legacy will live on and will likely save the lives of future generations of children unlucky enough to be born with MSD.

– Patrick O'Connor

WHY NOW?

We are at a critical moment for the development of treatments for rare and ultra-rare diseases. As new, transformative technologies like gene therapy are being tested in clinical trials to treat previously incurable genetic diseases like MSD, it's important that the wider healthcare community understand how these treatments work and how they fit into the overall rare disease landscape. The Zebra and the Bear can play an important role in educating the healthcare/ scientific community about gene therapy, but also about how a rare disease diagnosis can be devastating for families.

As Amber says in the film, "The truth is rare diseases are not in fact rare. There are over seven thousand of them, affecting millions of families." Which means that families around the world are experiencing the pain and heartbreak of what watching their children suffer. It is our intention to use this film to demonstrate what families shouldn't have to do in order to care for and ultimately save their children.

AN ULTRA-RARE DISEASE

"Rarest of the Rare"
About Multiple Sulfatase Deficiency (MSD) and Gene Therapy

MSD is a lysosomal storage disease, meaning the body does not break down and filter out the natural cellular waste that occurs in everyday cell functions. Children are typically without any symptoms at birth, but depending on their specific genetic variants, signs of MSD can begin either soon after children are born or later on in the child's life.

Multiple Sulfatase Deficiency can vary in severity, with more severe cases presenting earlier in life and progressing more quickly and with attenuated forms presenting later in life and progressing more slowly. It is important to remember that every child with MSD is unique and will follow their own path.

Historically, MSD has been presented as having three subtypes: Neonatal, Late-infantile, and Juvenile. The neonatal form is the most severe and can present in utero or at birth. These children decline very rapidly and often die during the first two years of life. Late-infantile is the most common form. Children with this form have normal cognitive development at the beginning of life but gradually begin to regress and lose skills. The final form, Juvenile, is the rarest. Individuals with this form typically do not show signs or symptoms until middle to late childhood and, generally, they have a slower loss of skills. (from the United MSD website)

GENE THERAPY

Gene therapy is a revolutionary medical technique that aims to treat or prevent diseases by modifying the genetic material within a patient's cells. It involves the introduction, removal, or alteration of genes to correct or counteract genetic disorders. Gene therapy can be performed using various methods, including:

- Gene Addition: Introducing new or modified genes into a patient's cells to replace or supplement faulty or missing genes.
- Gene Editing: Using technologies like CRISPR-Cas9 to precisely edit the DNA sequence at specific locations, correcting mutations directly.
- Gene Silencing: Turning off or reducing the expression of harmful genes through methods like RNA interference (RNAi).

These techniques can be delivered to target cells using vectors, such as viruses, which are engineered to carry the therapeutic genes without causing disease. Non-viral methods, like nanoparticles or direct injection of DNA, are also used.

Gene therapy holds immense potential for treating a wide range of genetic disorders, including inherited diseases like cystic fibrosis, hemophilia, and muscular dystrophy, as well as certain types of cancer and viral infections like HIV. Despite its promise, gene therapy also presents challenges, including ethical considerations, delivery efficiency, and potential side effects. Ongoing research and clinical trials aim to address these issues and expand the applications of gene therapy in modern medicine.

NIH BESPOKE GENE THERAPY CONSORTIUM

The Bespoke Gene Therapy Consortium (BGTC) is a public-private partnership under the Accelerating Medicines Partnership (AMP) program. Launched in October 2021, its mission is to streamline the development and delivery of gene therapies for rare diseases, which often affect too few people to be commercially viable.

BGTC focuses on adeno-associated virus (AAV) technology, a common and safe gene delivery method. The consortium aims to create a standard operational playbook for developing gene therapies, including streamlined templates, regulatory files, and uniform manufacturing processes. This approach is intended to make gene therapies more accessible and affordable for individuals with rare genetic disorders.

By fostering collaboration among the National Institutes of Health (NIH), the U.S. Food and Drug Administration (FDA), pharmaceutical companies, and nonprofit organizations, BGTC seeks to accelerate scientific and regulatory advances in the gene therapy field.

FILMMAKING TEAM

Patrick O'Connor - *Director / Producer*
Margaret McMullan - *Producer*
Amber Olsen - *Producer / Film Participant*
Misty Talley - *Editor / Post-Production Supervisor*
Matt Wagner - *Graphic Design*
Leslie-Anne Eades/LMNTL - *Impact Producer*
Alex Wiltz - *Visual Effects*

Original Score by **Eric Phillips**
Featuring **Sebastian Junger** on accordion
and original song *"The Zebra and the Bear"* by
Della Memoria/Emily Sholes Smith

FEATURING
Dr. Brian Kirmse • Dr. Steven Gray
Dr. Sean Ekins • Dr. Cat • P.J. Brooks

CREDITS

DIRECTED AND WRITTEN BY
Patrick O'Connor

PRODUCED BY
Patrick O'Connor, Margaret McMullan and
Amber Olsen

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Patrick O'Connor

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Clay Hardwick
Dathon Seeley
Jim Ball

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Beth Young
Nathan Willis
VOICE-OVER AUDIO RECORDING
Jesse Ramon Loya

ORIGINAL SCORE BY
Eric Phillips
Sebastian Junger - *Accordion*

MUSIC
"The Zebra and the Bear"
Written and recorded by Della Memoria
Performed by **Emily Sholes-Smith**
Courtney Blackwell - *Cello*

FEATURING
Amber Olsen
Tom Cannan
Willow Cannan
Kylee Cannan
Jenna Cannan
Dr. Brian Kirmse
Dr. Steven Gray
Dr. Sean Elkins
Dr. Cathleen Lutz

ALSO FEATURING
Shannon and Amera Marie Reed
Jocleyn Mayfield
Brianna Jackson
Lori Kilpatrick
Dr. Catherine Zimmerman
Dr. Maximiliano Presa
Emma and Hannah Arminio
Dr. Mauricio De Castro
Paul Roberts III
Julia Pierson
Dr. Berge Minassian
Dr. Kim Goodspeed

POST-PRODUCTION SUPERVISOR
Misty Talley

POST SOUND
Mixed, Sound FX and Design by Mike L. Pipgras

VISUAL EFFECTS
Alex Wiltz

TITLE DESIGN / POSTER DESIGN
Matt Wagner

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- The Rare Life Podcast
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- The Ryan Foundation
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- Ultragenyx
Megan Maack
-Childhood Dementia Initiative
Daniel DeFabio - Global Genes
Monique Molloy
- Orphan Disease Center
Effie Parks
- Once Upon A Gene Podcast
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- CureSPG50
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- Child Life On Call
Sharon Terry
- Genetic Alliance
Leslie Urdaneta
- National MPS Society
Lauren Weber
- The Washington Post
Kendyl Willox
- Senator Roger Wicker's Office

AND

United MSD Foundation
The Jackson Laboratory
University of Texas Southwestern Medical Center
University of Mississippi Medical Center
University of North Carolina - Chapel Hill