



PTC Therapeutics Patient Engagement

Malisa Rust
Patient Engagement Liaison, US
Neurology

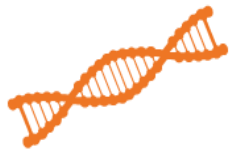
Improving patient outcomes by delivering **best-in-class therapies** earlier in disease progression



Earlier diagnosis



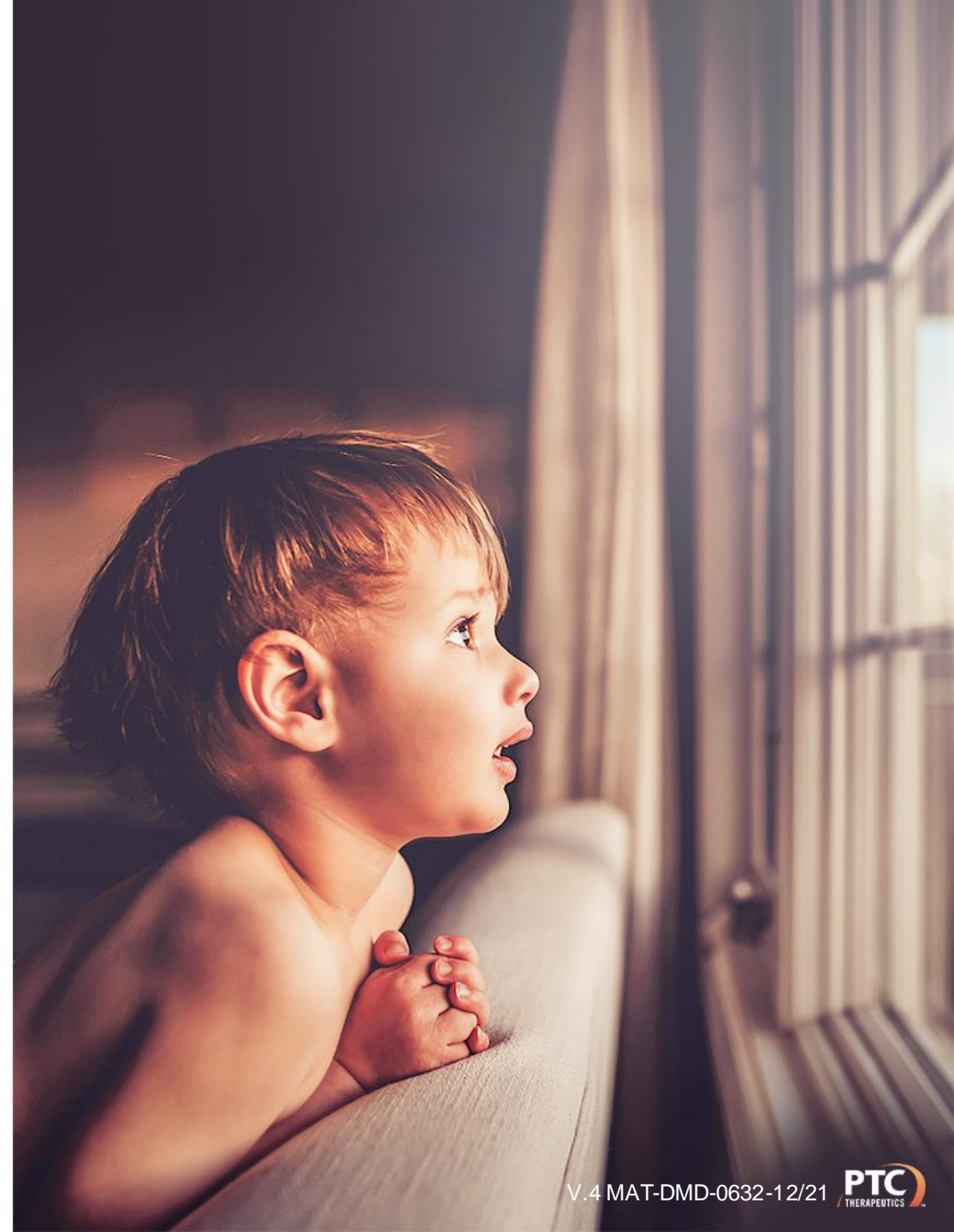
Increase disease awareness



Genotyping



Improving standards of care



PTC is committed to Duchenne families through **on-going programs**

Duchenne | and you SM

duchenneandyou.com

Register to stay up to date on news about Duchenne



SIBLINGS PROGRAM

First-in-kind program for siblings of Duchenne clinical trials



Annual Competitive Grant Program for Patient Advocacy Groups

Peer Navigator Program

For families considering corticosteroids

Phone: (866)282-5873

Email: peernavigator@ptcbio.com



Patient support program

1-844-478-2227

PTCCares.com

PTC Cares “in-house” support for the Duchenne community



Health Care Provider

- Initiates Prescription Start Form
- Works with PTC Cares to identify insurance coverage
- Provides continuous care



PTC Cares Hub - Single Point of Contact for Providers, Patients, and Specialty Pharmacy

- Verifies insurance benefits & assists with related issues
- Enrolls patient in appropriate assistance programs, if needed
 - Copay assistance-private insurance plans
 - Bridge program-for temporary lapse in coverage
 - Patient Assistance Program-for uninsured or no coverage
- Provides patient foundation information when appropriate
- Sends order to specialty pharmacy
- Assists with refills



Specialty pharmacy

- Dispenses/delivers drug
- Provides support services, education, 24/7 hotline



Patient/ Caregiver

- Initiates treatment
- Requests refill

For more information, call PTC Cares at 1-844-4PTC-CARES (1-844-478-2227)
Monday – Friday 8:00 a.m. a 6:00 p.m. or visit PTCCares.com

What is a Specialty Pharmacy?

Some prescriptions are available through a specialty pharmacy, which can play an important role.

They often provide:

- Advanced education, adherence, and support for rare and complex health conditions
- Unique storage and shipment requirements
- 24/7 access to specially trained pharmacists and clinicians
- Consultations to address patient or caregiver questions
- Work closely with the manufacturer/hub to support the patient and ship drug to their home

Specialty medications aren't like other drugs. They often are used in rare diseases and genetic disorders like **Duchenne Muscular Dystrophy**. Because there are so few people with certain rare diseases, it is shipped from a Specialty Pharmacy to their home versus trying to stock "brick and mortar" drugstores.



Unlike traditional "brick and mortar" drugstores, a specialized team of **pharmacists, nurses,** and **clinicians** are often on staff to support people who are using the medications.

Meet our Case Managers



Combined Skills

- Case Management Experience: 55 years
- Medical Billing & Coding: 48 years
- HCP Office Experience: 46 years
- Pharmacy Experience: 41 years
- Fluent Spanish Speakers: 2

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Neurology Patient Engagement Team

Brett Billmeyer
Head of PE



Location: Minnesota

Pat Cusick
PE Liaison



Location: Ohio

Cindy Salgado
PE Liaison



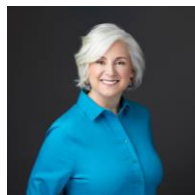
Location: California

Karina Lambertini
PE Liaison



Location: Florida

Patricia Brown
PE Liaison



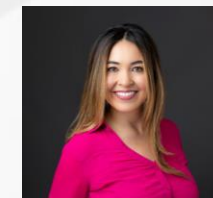
Location: Texas

Aurora Flores
PE Liaison



Location: California

Malisa Rust
PE Liaison



Location: Michigan

Caring for a loved one with Duchenne takes a team. **And it starts with you.**

You are the most important part of your child's care team. You see him every day. And you know him best, so you'll be the first to notice any changes in his health.

But you are not alone in caring for your child. You have a larger team behind you. This team may include family and friends. In addition to your child's primary healthcare provider, it also includes a group of healthcare specialists from different disciplines, like a neurologist, a cardiologist, and many others.



- Patient
- Family
- Neurologist
- Cardiologist
- Care Coordinator
- Emergency Physician
- Endocrinologist
- Gastroenterologist
- Geneticist
- Nutritionist
- Orthopedist
- Physical Therapist
- Psychologist
- Pulmonologist
- Social Worker
- Teachers



measured by moments

Everyone has a different definition of progress. For the last 20 years, we've measured our progress researching rare disease in moments. Smiling ones and crying ones. Moments spent with our boys' families and ones with their friends. We know that every step forward comes after several steps backward, because we've lived it—whether spending time with families in their homes or with our scientists researching in our labs.

It can be easy to lose yourself as you progress further. Although we've grown, our heart remains in the same place, because we've never measured ourselves like larger companies do. Our biggest accomplishment has always been the time we can give to all of our families. Whether it's hours, days, months, or years, every small moment is a big win.

THANK YOU!

Questions and information please contact:

Malisa Rust

U.S. Patient Engagement

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