



# PTC Therapeutics

Audrey Powell MD  
Senior MSL  
[apowell@ptcbio.com](mailto:apowell@ptcbio.com)

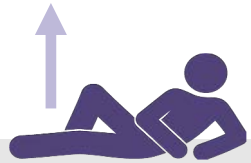
# Emflaza<sup>®</sup> (deflazacort) is the only corticosteroid approved in the US for DMD

- Emflaza<sup>®</sup> (deflazacort) is the only corticosteroid approved in the US for patients with DMD aged 2 years and older (US, 2017)<sup>1,2</sup>
  - It is provided as a tablet or suspension
  - The recommended once-daily dosage is 0.9 mg/kg/day (given orally)
- Deflazacort data demonstrate it is a best-in-class corticosteroid for the treatment of DMD<sup>3</sup>

DMD, Duchenne muscular dystrophy; US, United States. 1. US Food and Drug Administration. Emflaza<sup>®</sup> summary of product characteristics. Available from: [https://www.accessdata.fda.gov/drugsatfda\\_docs/label/2017/208684s000,208685s000lbl.pdf](https://www.accessdata.fda.gov/drugsatfda_docs/label/2017/208684s000,208685s000lbl.pdf) (accessed April 2021); 2. <http://ir.ptcbio.com/news-releases/news-release-details/ptc-therapeutics-receives-fda-approval-expansion-emflazar> (accessed April 2021); 3. McDonald CM, *et al.* *Muscle Nerve*. 2020;61(1):26–36

# Deflazacort was associated with statistically significant preservation of function across 3 key milestones in comparison to prednisone<sup>a</sup>

- Since 2006, the study enrolled over **440 patients** with DMD aged 2–28 years
- Patients were followed up for **up to 10 years**



2.1-year delay in age at loss of ability to stand from supine



2.7-year delay in age at LoA



2.7-year delay in age at loss of hand-to-mouth function with retained hand function (Brooke score  $\geq 5$ )

<sup>a</sup>Prednisone cohort was composed of prednisone/prednisolone users.

P values for difference in age at loss of ability to stand from supine, age at LoA and age at loss of hand-to-mouth function with retained hand function were: 0.0114, 0.0102 and 0.0110, respectively. DMD, Duchenne muscular dystrophy; LoA, loss of ambulation.

McDonald CM, *et al. Lancet.* 2018;391(10119):451–461.

# Overall summary



Deflazacort is the **only approved** corticosteroid for DMD in the US



Deflazacort forms part of the **standard of care** and will continue to be a mainstay of therapy for DMD



Deflazacort has a **more beneficial immunomodulatory profile** which may support greater muscle preservation compared to prednisone\*



A substantial and consistent body of evidence has demonstrated the **treatment benefits of deflazacort** as compared to prednisone\*

\*Prednisone or prednisolone. DMD, Duchenne muscular dystrophy.

# Ataluren overview

- Conditionally approved in Europe for ambulatory patients 2 years and older with DMD caused by a nonsense mutation\*
- Ataluren *bypasses* the nonsense mutation and allows for formation of a full-length, functional dystrophin protein
- Available to boys in over 40 countries through commercial and PTC-ACTs\*
- Active discussions with the FDA regarding approval in the US
  - A clear pathway forward has been provided
  - Additional clinical study required
  - Resubmission upon completion of clinical study
- Ataluren is considered an investigational product in the US

\*Translarna ▼ (ataluren) is indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 2 years and older in the European Member States and Iceland, Liechtenstein, Norway, Great Britain, Northern Ireland, Kazakhstan, Belarus, Israel, and Republic of Korea and Russia, and aged 5 years and older in Chile, Brazil, and Ukraine (under special state registration). In Brazil, the indication is restricted to pediatric male patients. The presence of a nonsense mutation in the dystrophin gene should be determined by genetic testing.<sup>1,2</sup>

<sup>1</sup>Translarna Summary of Product Characteristics (SmPC).

<sup>2</sup>Translarna Instructions for Use [IFU] – Russia.



A photograph of a man and a young boy. The man is in the foreground, looking up and smiling. The boy is leaning over his shoulder, resting his head on the man's face and smiling. The background is a blurred indoor setting with green plants. A semi-transparent teal overlay covers the right side of the image, containing white text.

**PTC is  
Committed  
to Duchenne  
Families**

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

**Duchenne** | and you <sup>SM</sup>

**[duchenneandyou.com](http://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](mailto:peernavigator@ptcbio.com)*



*Patient support program*

*1-844-478-2227*

*[PTCCares.com](http://PTCCares.com)*