



IN THE TREATMENT OF DUCHENNE MUSCULAR DYSTROPHY (DMD)

**THE STANDARD OF CARE HAS
COME A LONG WAY.
TOGETHER, CAN WE DO MORE?**





WANTING STRONGER MUSCLES AND BONES IS SOMETHING WE ALL CAN AGREE ON.

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Glucocorticoids can help strengthen muscles and may delay the loss of walking and other abilities for people with Duchenne muscular dystrophy (DMD). While glucocorticoids significantly advance care, a number of issues including weight gain, bone health, growth delays, and behavioral changes still remain.¹

DMD HAS CONSEQUENCES THROUGHOUT THE BODY.

DMD is characterized by progressive muscle loss and weakness. Beyond muscles, DMD has an effect on patients' heart health, breathing ability, and skeletal health.¹

DETERIORATION OF BONE HEALTH IS ONE OF THE MANY SERIOUS CONSEQUENCES OF DMD.

Bone development is partly dependent on the action of muscles. The shape and thickness of bones is influenced by the mechanical force muscles put on them.²

This relationship is disrupted by DMD. As a result, bone fragility is a major component of disease progression. People with DMD can experience a progressive loss of bone mass (osteoporosis) and demineralization (osteopenia) that lead to bone fractures and abnormal bone development.^{3,4}

Early intervention is critical: A delayed start of therapeutic interventions may lead to faster loss of muscle strength, increased bone fragility, the onset of contractures, reduced mobility, and shorter lifespan.^{3,5,6}

DMD IMPACTS BONE HEALTH IN A NUMBER OF WAYS⁷:

- Muscle weakness and immobility
- Activation of bone-destroying cells
- Lack of vitamin D
- Insufficient calcium maintenance

MANAGING SKELETAL SYMPTOMS OF DMD^{4,8}:

- Standing, even for short periods of time, can help strengthen bones
- Routine physical therapy can help maintain joint flexibility and relieve stiffness
- Curving of the spine (scoliosis) can occur and may require surgical correction
- Bisphosphonates can help to strengthen bone

GLUCOCORTICOIDS DO A LOT OF GOOD. COULD THEY DO MORE?



EFFECTIVE TREATMENTS ARE NEEDED THAT PROVIDE THE EFFICACY OF TRADITIONAL GLUCOCORTICOIDS WHILE POTENTIALLY REDUCING SELECT SIDE EFFECTS.

Traditional glucocorticoids are the foundational treatment in Duchenne muscular dystrophy (DMD).⁹

This treatment has been shown to **slow the start of DMD symptoms**. Glucocorticoids may help delay the loss of muscle strength, muscle function, and walking ability. They are also believed to help maintain heart health.^{1,6,10}

Despite their benefits, glucocorticoids carry many potential side effects for people with DMD.

SIDE EFFECTS AND SAFETY CONCERNS COMMONLY ASSOCIATED WITH TRADITIONAL GLUCOCORTICOIDS INCLUDE¹¹:



Weight gain



Poor bone health



Growth failure or delay



Behavioral changes



Cushingoid appearance

TRADITIONAL GLUCOCORTICOIDS INCREASE RISKS OF BONE FRAGILITY AND FRACTURES.

In a study of 408 people (ages 3-19 years) with DMD treated with glucocorticoids⁷:



Most individuals experienced at least one fracture by age 18



Compression fractures of the spine occurred in nearly 60% of study participants by age 18

MONITORING BONE HEALTH WITH IMAGING AND BIOMARKERS IS ESSENTIAL FOR ALL PEOPLE WITH DMD USING TRADITIONAL GLUCOCORTICOIDS

TALK TO YOUR CARE TEAMS ABOUT MANAGING AND MONITORING BONE HEALTH

Bone health biomarkers and regular imaging should be part of all DMD care plans. Help your doctors by reporting any changes to your health, including feelings of pain.⁴

LEARN MORE ABOUT THE BENEFITS AND CHALLENGES OF DMD'S CURRENT STANDARD OF CARE

Visit [DoMoreForDMD.com](https://www.domorefordmd.com)



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