



# LIFE TAKES MUSCLE

STOP **SMA** FROM DOING THE SAME

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**A guide to unmet needs and a muscle-focused future  
in spinal muscular atrophy (SMA)**

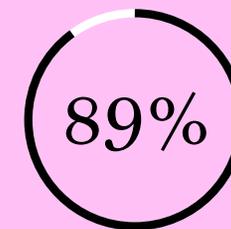
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# **PEOPLE LIVING WITH SMA WANT MORE**

By definition, spinal muscular atrophy (SMA) is a *neuromuscular* disease that results in a loss of both nerve cells and muscle. Recent treatments have made great strides in treating people living with SMA, but people living with SMA continue to look for more—more muscle strength, more motor function, stability, and more out of life.

**In a Cure SMA survey of over 300 participants, the top thing people living with SMA wanted was MORE MUSCLE STRENGTH.\***



cited gaining muscle strength as the most important unmet need they hope new therapies will address

\*Data from 2024 Cure SMA Community Update Survey of people with SMA and caregivers (n=311).

Visit [LifeTakesMuscle.com](https://www.lifetakesmuscle.com) to learn how the SMA community is putting a focus on muscle

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# MAINTAINING MUSCLE IS IMPORTANT

Approved SMN-targeted treatments focus on motor neuron loss, but the role of muscle can't be overlooked.

## Did you know?

There are multiple kinds of muscles:

- a** **Smooth muscles** control involuntary functions such as digestion and blood circulation
- b** **Cardiac muscles** control the rhythmic contractions that pump blood throughout the body
- c** **Skeletal muscles** are attached to bones throughout the body, enabling voluntary movements

**SMA primarily impacts voluntary functions controlled by skeletal muscles**—which includes almost any movement we make, and even some essential physiological functions many people take for granted, like breathing.



**Lyza, 23**

Living with SMA

On an approved SMN-targeted treatment for 8 years

Lyza transferring from her chair to her bed

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**Mckenna, 16**

*Living with SMA  
On an approved SMN-targeted  
treatment for 8 years*

# MUSCLE PLAYS A CRITICAL ROLE IN EVERYDAY LIFE

Muscles enable us to do things like brush our teeth, lift a glass, or turn pages in a book—the little things that are often called “activities of daily living” that can help foster independence in ourselves and our loved ones.

They enable us to function in ways that are important to our social and emotional well-being. And muscles are important to many essential physiological functions we may take for granted, like breathing, eating, and speaking.

## Did you know?

**9 muscles** work together when you smile

**12 muscles** are working together during a chair transfer

**52 muscles** are at work when you breathe

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# FATIGUE CAN BE DEBILITATING IN SMA

For people living with spinal muscular atrophy (SMA), fatigue is a part of everyday life. It's one of the most debilitating and under-addressed aspects of SMA. **In one survey, 71%** of people living with SMA said reducing fatigue was one of the most significant unmet needs that they hoped a new therapy would address.\*

\*Data from the 2024 Cure SMA Community Update Survey.

## Working Around Limitations

Because progressive muscle weakness and fatigue are so common and can have such an impact on daily life, many people with SMA develop their own adaptations or workarounds.

**These workarounds generally fall into two categories:**

**Physical Workarounds,** which include assists from an outside force

**Behavioral Workarounds,** which are strategies used to outsmart fatiguing activities



**Jeybriel, 3**

Living with SMA  
On an approved SMN-targeted  
treatment for 1 year

## **SEEING SMA THROUGH A NEW LENS**

Today, approved medications focus on increasing production of survival motor neuron (SMN) protein to help people with spinal muscular atrophy (SMA) live longer and improve motor function. But these treatments don't directly address the muscle. As time passes and SMA progresses, it's not just motor neurons that are lost. Muscles often continue to atrophy, causing motor function to decline and creating a downstream impact on daily living.

### **A FUTURE FOCUSED ON MUSCLE**

The future of SMA treatment will build on SMN-targeted therapies in hopes of gaining both muscle strength and motor function. Not only would this potentially help address progressive muscle weakness, but it could also impact quality of life.



**Bear, 10**

*Living with SMA*

*On an approved SMN-targeted treatment for 9 years*

## **MYOSTATIN: A POTENTIAL TARGET IN SMA**

**Myostatin** is a protein found in everyone's body. Its job is to inhibit muscle growth, or act as a natural brake to keep growth in check. Though it is not a cause of neuromuscular diseases like SMA, scientists are studying whether treatments targeting myostatin can help address progressive muscle weakness in SMA.

### **WHAT CAN WE LEARN ABOUT MYOSTATIN FROM THE BELGIAN BLUE COW?**

The Belgian Blue breed of cattle has a natural mutation in the myostatin gene, which leads to unchecked muscle growth.

In SMA, where an important goal is to stop or even reverse muscle weakness, myostatin has understandably attracted significant interest.

Research continues to look for optimal ways to specifically target myostatin to help address muscle weakness.

Due to a mutation in the myostatin gene, the Belgian Blue Cow has inspired research in SMA



# STRENGTHENING CONVERSATIONS WITH YOUR DOCTORS

You are your own best advocate. And your treatment team is in your corner. But they're only working with the information you give them about you or the person you care for.

## BEFORE YOUR APPOINTMENT

Write out answers to the questions below. Talk through these answers with a caregiver, friend, or loved one. If they're coming with you to the appointment, they'll offer added assurance that all your concerns are addressed and provide a second set of ears for the conversation that follows.

### My next appointment

Date: \_\_\_\_\_ Time: \_\_\_\_\_

Whom from your treatment team do you want to have a conversation with?

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What are your treatment goals?

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What function do you want to maintain or gain to achieve your goals?

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What are you worried about losing?

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# TALK TO YOUR DOCTOR ABOUT **MYOSTATIN** AND YOUR **SMA GOALS**

Keep up with Life Takes Muscle

Sign up at [LifeTakesMuscle.com/sign-up](https://LifeTakesMuscle.com/sign-up) to receive updates on developments in the SMA treatment landscape, hear about the community's evolving goals, and learn more about the need to preserve and strengthen muscle.

Follow us @lifetakesmuscle

