

A new tool for tracking the progression of spinal muscular atrophy (SMA): quantitative magnetic resonance imaging (qMRI).

A Roche collaboration study with the University of Basel Children's Hospital

Key points

- The imaging test, quantitative magnetic resonance imaging (qMRI), could see differences between people with spinal muscular atrophy (SMA) who could walk and unaffected individuals in this study.
- The qMRI data correlated well with measures of mobility and movement.
- qMRI may have potential as a biomarker for SMA progression in clinical trials.

What was this study?

Researchers examined ways of tracking change in people with Type 3 SMA over a 1-year period using different biomarkers.¹ They also looked at whether these biomarkers correlated with well-established measures of mobility and movement (MFM [motor function measure] and 6MWT [6-minute walk test]).

This study looked at 19 people with Type 3 SMA who were able to walk and 19 unaffected healthy individuals. They were aged between 11 and 60 years.

The study took place at the University Children's Hospital in Basel, Switzerland in collaboration with Roche.

What did qMRI imaging show?

qMRI could clearly identify people with SMA versus unaffected individuals.

qMRI images also showed that people with SMA had reduced thigh muscle size, and higher fat-to-muscle ratio compared with the healthy volunteers.

However, qMRI did not show any change in the muscle of people with Type 3 SMA over the 1-year period of the study. People with Type 3 SMA are known to progress slowly, with little change year-to-year.

How did the qMRI finding relate to other measures?

Muscle size correlated well with established measures of movement (MFM and 6MWT). This suggests the muscle properties seen by qMRI are closely linked to movement and mobility. Both measures of movement (MFM and 6MWT) showed little change over 1 year.

What does this mean for people with SMA?

qMRI may have potential as a biomarker in SMA clinical trials complementing established measurements such as MFM and 6MWT.

This imaging technique, which correlated well with MFM and 6MWT, could potentially monitor muscle composition in people with SMA.

As disease progression in milder forms of SMA is slow and difficult to detect, qMRI could potentially help track change over time. However, the findings of the study suggest that 1 year is not long



Example images showing a cross-section of the thigh

enough to detect change. Therefore, SMA clinical trials may need to run for longer than 1 year to show potential beneficial effects of investigational medicines.

Background

What are biomarkers?

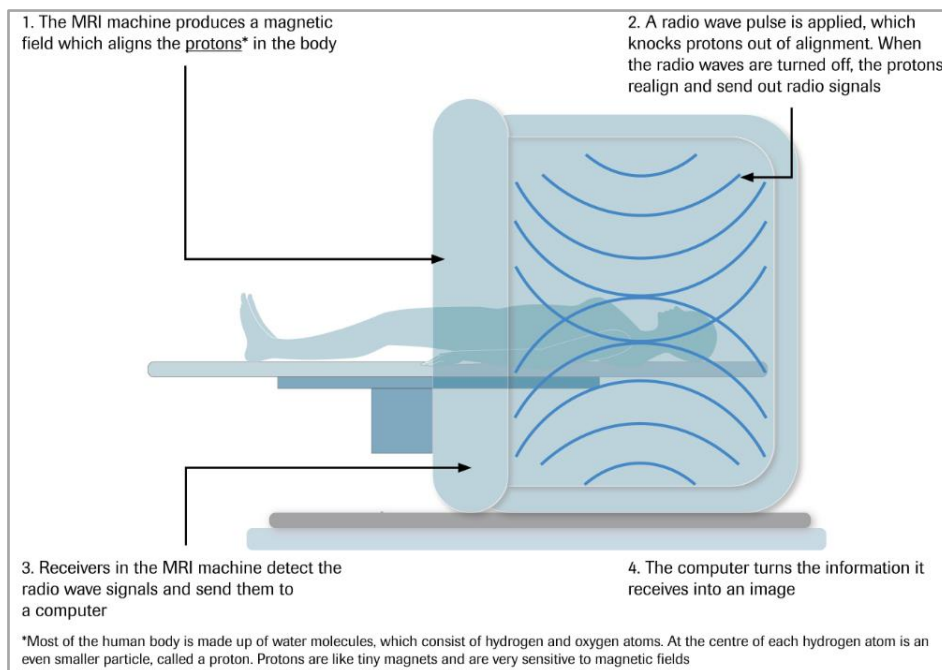
Biomarkers are molecules or biological characteristics that can be measured in cells or tissues. Biomarkers serve as indicators of the presence or progress of disease, or the effects of treatment. For example, cholesterol level in the blood is a biomarker used to determine the risk of heart disease.

SMA progression can be slow and difficult to measure, particularly for later-onset forms, such as Type 3. Whilst many biomarkers have been assessed in people with SMA, there is no biomarker that is widely used to reliably measure SMA progression or the potential benefits of new treatments.

What is magnetic resonance imaging (MRI)?

MRI is a safe and painless technique that uses a magnetic field and radio waves to create computer-generated detailed images of tissues and organs² (see figure³ below). Unlike measures of movement, qMRI is not affected by a person's level of tiredness or motivation.

These qMRI images can show changes like loss of muscle and how much muscle has been lost. This could help monitor SMA worsening over time and even be used in clinical trials to monitor the potential benefit of investigational medicines.



What is the MFM and 6MWT?

- The Motor Function Measure (MFM) scores the amount of movement and mobility achieved.
- The 6-Minute Walk Test (6MWT) measures the distance walked in 6 minutes.

Both tests require the person to do certain exercises. Therefore, the score can be affected if someone is tired or not motivated on the day.

References

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