

SMA Priority Setting Partnership project

List of the Top 10 unanswered research questions as voted per PSP process

- Can nerve cells (especially motor neurons) be regenerated in people with SMA?
- Are there biological markers (biological molecules in blood or other tissues) that can indicate the progression of SMA?
- Can damaged muscles be rebuilt in people with SMA? How can muscles be strengthened?
- Do people with SMA benefit from a specialised diet (e.g. amino acid diet) or dietary changes such as treatment with supplements?
- What is the best physiotherapy approach for people with SMA? Does it need to be tailored to the individual (e.g. according to age and type of SMA)? Does it need to change during treatment with one of the new drugs?
- What parts of the body are affected by SMA other than nerves and muscles?
- Does electrical stimulation of nerves and /or muscles benefit people with SMA?
- What is the best approach to treating and preventing problems with bones and joints (orthopaedic problems) for people with SMA?
- What are the best ways to treat and manage fatigue in people with SMA?
- How can new technology and assistive devices help people with SMA and their carers?