

An Update on Scholar Rock's Progress in SMA

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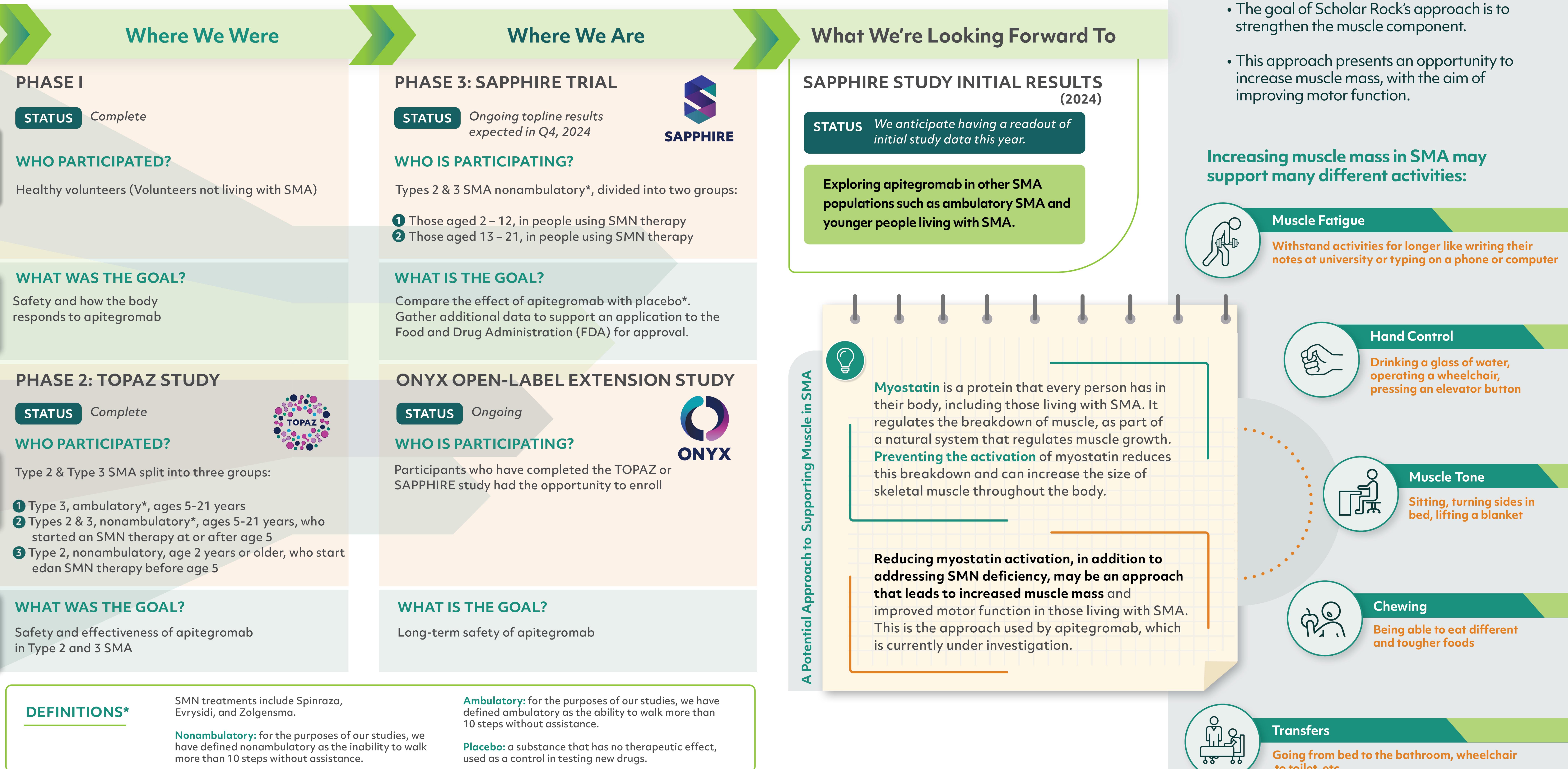


Introduction

Currently approved SMN treatments* have helped those living with SMA by slowing motor neuron degeneration, however muscle atrophy and associated weakness remains a continued challenge for many.

Scholar Rock is researching apitegromab, which aims to address muscle atrophy and associated weakness in SMA. After several years of clinical studies, Scholar Rock is anticipating having phase 3 clinical study results in 2024.

Apitegromab is an investigational treatment and the efficacy and safety have not been evaluated by the U.S. Food and Drug Administration (FDA). If successful, Scholar Rock intends to submit an application to the FDA for approval.



DEFINITIONS*

SMN treatments include Spinraza, Evrysidi, and Zolgensma.

Nonambulatory: for the purposes of our studies, we have defined nonambulatory as the inability to walk more than 10 steps without assistance.

Ambulatory: for the purposes of our studies, we have defined ambulatory as the ability to walk more than 10 steps without assistance.

Placebo: a substance that has no therapeutic effect, used as a control in testing new drugs.