Who Is Scholar Rock And What Is SRK-015?

Some of the information below was copied verbatim from the SMA News Today article & Scholarrock.com

Based in Cambridge, Massachusetts, Scholar Rock is discovering and developing a pipeline of innovative new medicines to treat a range of serious diseases with high unmet medical needs, including neuromuscular disorders, cancer, fibrosis, and anemia.

SRK-015, their most advanced drug candidate, is a selective and local inhibitor of latent myostatin. Scholar Rock is developing and investigating their drug as a treatment to improve muscle strength and motor function in patients with Spinal Muscular Atrophy (SMA).

Myostatin is a member of a superfamily of growth factors and is expressed primarily in skeletal muscle cells to inhibit muscle growth. In the body, it works in concert with other growth factors and hormones to maintain appropriate muscle mass. There has been an emerging interest in therapeutically targeting myostatin following the discovery of myostatin-deficient animals that have increased muscle mass and strength.

Who Is Scholar Rock And What Is SRK-015?

Some of the information below was copied verbatim from the SMA News Today article & Scholarrock.com

SRK-015 uniquely targets the latent form of myostatin, specifically blocking its activation in muscle. Inhibiting the supracellular activation of myostatin, rather than the traditional approach of blocking already activated, mature myostatin or the myostatin receptor, avoids blocking the activity of other closely-related members of the superfamily that may lead to undesirable side effects.

Scholar Rock is advancing their new drug, a first-in-class selective inhibitor of the activation of myostatin, into clinical development for the treatment of SMA. They have initiated a Phase 1 clinical trial and intend to commence a Phase 2 trial in patients with later-onset SMA in the first quarter of 2019. They believe that their drug has the potential to be the first muscle-directed therapy to reverse or prevent muscle atrophy in SMA patients and could be used both as a monotherapy or together with the current standard of care.

Who Is Scholar Rock And What Is SRK-015?

Some of the information below was copied verbatim from the SMA News Today article & Scholarrock.com

Excessive activation of myostatin is associated with muscle atrophy. Prior trials of compounds targeting the mature form of myostatin found that they can affect other closely related growth factors, possibly causing side effects.

In preclinical studies, their drug prevented additional atrophy in mice with muscle wasting and increased healthy animals' muscle mass and function.

Nagesh Mahanthappa, president and CEO at Scholar Rock, said the following in a recent press release: "The initiation of this Phase 1 clinical trial of SRK-015 is a momentous milestone for Scholar Rock and we are one step closer to potentially bringing the first muscle-directed therapy to patients suffering from SMA."

Who Is Scholar Rock And What Is SRK-015?

Some of the information below was copied verbatim from the SMA News Today article & Scholarrock.com

The placebo-controlled, double-blind Phase 1 study is assessing the safety, tolerability and pharmacological profile of single and multiple intravenously ascending doses of their drug in healthy adult volunteers.

If successful, Scholar Rock plans to open a Phase 2 study early next year to test the efficacy and safety of their drug in patients with later-onset SMA.

The therapy will be assessed either as a stand-alone therapy or in combination with an approved SMN therapy as a background standard of care.

The U.S. Food and Drug Administration granted SRK-015 orphan drug status for the treatment of spinal muscle atrophy, and in January 2018, Scholar Rock announced $47 million in new financing for the compound's clinical testing.

The Phase 1 trial is continuing to enroll healthy volunteers, but more information about the trial, including test location(s), was not available.

My Opinions Regarding Scholar Rock And SRK-015

Scholar Rock’s recent announcement of their new drug, is just another example of the attention SMA is receiving in the medical world. Those of us sufferer from SMA have seen medical establishments and pharmaceutical companies scrambling to bring more treatment options to the forefront of battling this deadly disease. After Biogen released the first FDA approved treatment for SMA called Spinraza, it seems as if Pandora's box had been opened, and a wide variety of new drugs and potential treatments started becoming available.

My Opinions Regarding Scholar Rock And SRK-015

While it's still too early to start anointing this new drug as a potential treatment, it's not too early to raise the hopes of those of us suffering from SMA. Two years ago, those of us who suffer from SMA had nothing to look forward to. If Scholar Rock is successful in getting their new drug all the way through the FDA approval process to make it an actual treatment, this could be one more medicine that could not only prevent the devastating results that SMA causes, it could possibly reverse some of the damage that those of us with SMA have already suffered. In my opinion, this is just another example of the amount of attention that is happening within the medical establishment, thus, increasing our excitement as patients. One step of hope equates to a large leap of excitement for those of us who, just a few years ago, had nothing to be excited about.