Novartis Pharmaceuticals

All information was copied verbatim from Wikipedia (Link in Description of video)

Novartis International AG is a Swiss multinational pharmaceutical company based in Basel, Switzerland. It is one of the largest pharmaceutical companies by both market cap and sales.

Novartis AG owns, directly or indirectly, all companies worldwide that operate as subsidiaries of the Novartis Group.

Novartis AG also holds 33.3% of the shares of Roche however, it does not exercise control over Roche. Novartis also has two significant license agreements with Genentech, a Roche subsidiary.

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About Branaplam:

Branaplam, also known as LMI070 and NVS-SM1, is a highly potent, selective and orally active small molecule experimental drug being developed by Novartis to treat spinal muscular atrophy (SMA). It is a pyridazine derivative that works by increasing the amount of functional survival of motor neuron protein produced by the SMN2 gene through modifying its splicing pattern. As of March 2017, branaplam is in a phase-II clinical trial in children with SMA type 1.

Novartis Pharmaceuticals

All information was copied verbatim from the Cure SMA website (Link in Description of video)

About Novartis – LM1070

Novartis has started a new clinical study to evaluate LMI070, a novel investigational oral compound, for the potential treatment of infants with Type I spinal muscular atrophy (SMA). LMI070 treatment in animals prolonged survival and increased the amount of functional SMN protein due to more efficient splicing of the SMN2 gene. LMI070 is being tested to determine if can produce improved motor neuron survival, muscle growth, motor milestones, and respiratory function in infants with Type I SMA.

This First in Human study (NCT02268552) will evaluate the safety and tolerability of LMI070 in Type 1 SMA patients, and explore the potential therapeutic benefit in patients. This is an open label study; all patients will receive LMI070. Patients will receive LMI070 for 13 weeks. An increase in the dose of LMI070 and continuation of LMI070 treatment beyond the initial 13 weeks may be possible. To participate in the study, patients must be between 1-7 months of age when they enroll and must have two copies of the SMN2 gene.

Novartis Pharmaceuticals

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Novartis Launches Phase 2 Clinical Trial

BY CURE SMA | PUBLISHED ON JUNE 3, 2015

Novartis has published a paper in the journal Nature Chemical Biology, detailing promising results in their program to develop an orally available SMA drug that corrects SMN2 splicing. In testing, the drug increased SMN protein levels and extended survival in a severe mouse model of SMA.

Novartis is currently testing this drug in a Phase 2 clinical trial at several European sites. The open-label trial is enrolling infants aged six months or less with SMA type I, who also meet other inclusion/exclusion criteria. Complete information on this clinical trial can be found on the FDA's clinical trial registry.

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Individuals with SMA don't produce SMN protein at high enough levels, due to a mutation in the SMN1 gene. Individuals with SMA do have one or more copies of the SMN2 gene, which also produces SMN protein, but most of the protein produced by SMN2 lacks a key building block.

Cure SMA has identified four possible therapeutic approaches for treating SMA, one of which targets the SMN2 gene. The goal of this approach is to correct the gene splicing, meaning SMN2 could produce a complete protein, or to prompt SMN2 to make more protein. The Novartis program is one of several programs pursuing this approach.

Novartis Ongoing Clinical Trial

All information was copied verbatim from the ClinicalTrials.gov website (Link in Description of video)

An Open Label Study of LMI070 (Branaplam) in Type 1 Spinal Muscular Atrophy (SMA) An open-label, multi-part, first-in-human study of oral branaplam in infants with Type 1 spinal muscular atrophy. The purpose of this study is to evaluate the safety, tolerability, pharmacokinetics (PK), pharmacodynamics (PD) and efficacy after 13 weeks; and to estimate the Maximum Tolerated Dose (MTD) of orally administered branaplam; and to identify the dose that is safe for long term use as well as that can provide durable efficacy optimal dosing regimen in patients with Type 1 SMA.

The trial identifier is NCT02268552 **Estimated Enrollment: 44 participants**

Actual Study Start Date: April 2, 2015
Estimated Primary Completion Date: November 14, 2019
Estimated Study Completion Date: November 14, 2019
Ages Eligible for Study: Up to 182 days old (Child)

Pharmacokinetics (PK) is the study of how an organism affects a drug, whereas pharmacodynamics (PD) is the study of how the drug affects the organism. Both together influence dosing, benefit, and adverse effects.