Pharmaceutical Companies Active In SMA Treatments

I will be examining one pharmaceutical company in this presentation that is currently pursuing an SMA treatment within the end of 2018 and first quarter of 2019. In upcoming videos, I will examine other pharmaceuticals that are also active in getting their treatment to us in the near future.

While I fully understand that these treatments are at least one year away from becoming reality, this is the time that we, as SMA patients should take, to begin familiarizing ourselves with these pharmaceutical companies and what they could possibly have for us within the next few years.

The pharmaceutical companies that I will be looking at in the upcoming months, will be those companies that I believe have the greatest chances of getting their treatments passed through the U.S. Food and Drug Administration (FDA). Much of the information that I'm going to be giving you was information that was given to me by the pharmaceutical company itself, or information that I found either on their website or on the Internet itself. Any and all graphics used in my presentation will be linked in the description field of this video so that my subscribers and followers can go to these websites and see this information for themselves.

AveXis (AVXS -101)

AveXis is a gene therapy company that is focused on bringing gene therapy from the lab to the clinical setting for patients and families with rare and orphan neurological genetic diseases. Their initial proprietary gene therapy candidate, AVXS-101, is being evaluated for the treatment of Spinal Muscular Atrophy (SMA) Type 1 and Type 2.

They have been granted proprietary gene therapy status for their development of AVXS-101, which has been granted Orphan Drug Designation for the treatment of all types of SMA, and Breakthrough Therapy Designation, as well as Fast Track Designation, for the treatment of SMA Type 1 which is one of the most life-threatening neurological genetic disorders in children.

Their primary focus is to develop gene therapies for SMA, initially targeting SMA Type 1 – which is currently in the development stage. They have recently initiated a Phase 1 clinical trial to investigate AVXS-101 for the treatment of SMA Type 2. ***Data from this study will help inform potential future development plans in other SMA subtypes, SMA Type 3 and Type 4.***

AveXis (AVXS -101)

SMA Type 1 Pivotal Trial (Copied Verbatim)

The open-label, single-arm, single-dose, multi-center trial – known as STR1VE – is designed to evaluate the efficacy and safety of a one-time IV infusion of AVXS-101 in patients with SMA Type 1. The co-primary efficacy outcome measures of the trial include the achievement of independent sitting for at least 30 seconds at 18 months of age; and, event-free survival at 14 months of age. Co-secondary outcome measures include the ability to thrive, and the ability to remain independent of ventilatory support at 18 months of age.

AveXis (AVXS -101)

SMA Type 2 Phase 1 Trial (Copied Verbatim)

The open-label, dose-comparison, multi-center Phase 1 trial – known as STRONG is designed to evaluate the safety, optimal dosing, and proof of concept for efficacy of AVXS-101 in two distinct age groups of patients with SMA Type 2, utilizing a one-time IT route of administration. The primary outcome measure for patients less than 24 months of age at the time of dosing is the achievement of the ability to stand without support for at least three seconds. The primary outcome measure for patients between 24 months and 60 months of age at the time of dosing is the achievement of change in Hammersmith Functional Motor Scale Expanded from baseline. The secondary outcome measure for both age groups is the proportion of patients that achieve the ability to walk without assistance, defined as taking at least five steps independently while displaying coordination and balance. Developmental abilities, including motor function, will also be evaluated as exploratory objectives.

Gene Replacement Therapy Clinical Trial for Patients With Spinal Muscular Atrophy Type 1 (STR1VE)

ClinicalTrials.gov Identifier: NCT03306277

Recruitment Status : Recruiting
First Posted : October 11, 2017
Last Update Posted : January 17, 2018

See Contacts and Locations

NIH U.S. National Library of Medicine
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Study of Intrathecal Administration of AVXS-101 for Spinal Muscular Atrophy (STRONG)

The safety and scientific validity of this study is the responsibility of the study sponsor and investigators. Listing a study does not mean it has been evaluated by the U.S. Federal Government. Know the risks and potential benefits of clinical studies and talk to your health care provider before participating. Read our disclaimer for details.

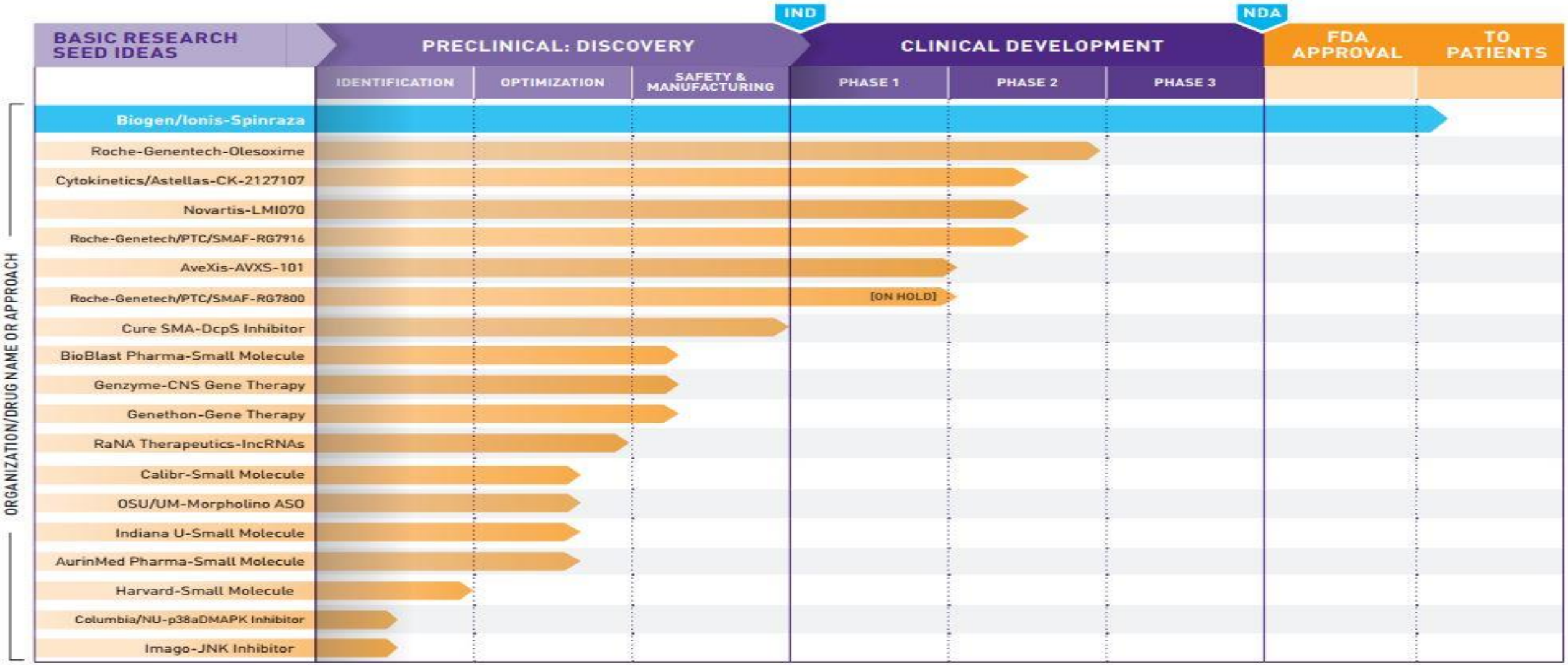
ClinicalTrials.gov Identifier: NCT03381729

Recruitment Status : Recruiting
First Posted : December 22, 2017
Last Update Posted : December 22, 2017

See Contacts and Locations

SMA DRUG PIPELINE

This year, we are funding research with more breadth, depth, and diversity than ever before. This chart shows the drugs and therapies that are currently in the pipeline for SMA.



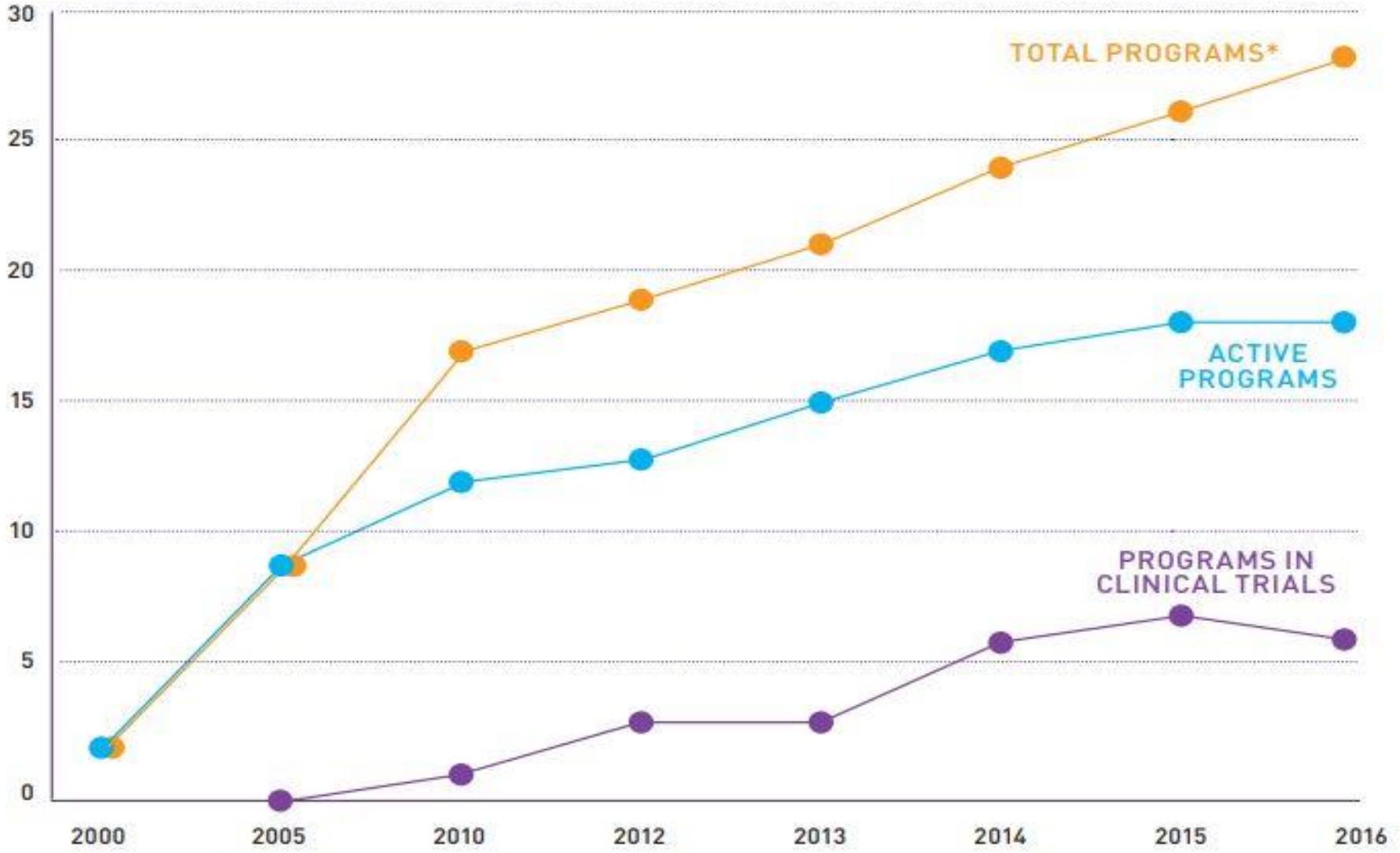
IND = Investigational New Drug

NDA = New Drug Application

Last updated: January 2017

cure SMA

HOW THE PIPELINE HAS GROWN



*Includes failures

NUMBER OF COMPANIES INVESTING IN SMA DRUG PROGRAMS