



Ten years of Partnership in Spinal Muscular Atrophy (SMA)

Roche

In 2021, Roche celebrated 10 years of working in partnership with the SMA community to achieve our common goal of improving the lives of people affected by this rare condition.

The story so far...



1

2011

Roche enters existing collaboration between the SMA Foundation and PTC Therapeutics, with the goal of converting PTC's cutting-edge technology into a life-saving treatment for people with SMA^{1,2}



Partnership excellence has been key to the SMA programme, with each partner contributing their unique capabilities and skills to the overall mix

Senior Vice President, Roche Global Head Neuroscience and Rare Diseases Clinical Development²



2

2011-2015

Scientists from the three partners study several promising molecules with the potential to treat SMA and make a meaningful difference to those living with the condition^{2,3}



In scientific research, failure can come at any time but it cannot define you. It was disappointing when our first candidate was stopped, but... Our management and environment made it safe to be courageous and try again – even to fail again, although luckily we did not!

Roche Medicinal Chemist³

3

2015

Scientists determine that some investigational molecules are unlikely to be safe and effective, but one promising molecule does emerge³⁻⁶



4

2016

Roche creates the broadest pivotal SMA clinical development programme to date, with patients of different ages, types and severity, to investigate this promising molecule⁷⁻¹¹



The programme, supported by our partners and designed with the patient and clinical community, includes:

- People with different types of SMA, from birth to 60 years old
- Pre-symptomatic people and those previously treated with other SMA-targeted therapies
- People underrepresented in other SMA studies and from countries not typically included in clinical trials⁷⁻¹¹

5

October 2017



SMA Independence Scale (SMAIS) developed in collaboration with Cure SMA, SMA Europe and people living with SMA¹²

Developed in response to community need to maintain independence, it is a clinical tool that measures the level of support required to perform smaller daily activities with SMA¹²

SMAIS is available to all researchers and healthcare professionals free of charge



6

2018

Roche joins the Cure SMA Clinical Trial Readiness Program¹³

Created a new means for:

- Engaging research sites interested in SMA trials
- Providing tangible resources to optimise trial readiness
- Promoting patient-centred trial management¹³



7

2018

Roche joins the US Newborn Screening Coalition to campaign for newborn screening to include an SMA test¹⁴

Evidence shows that early diagnosis and treatment of SMA leads to better outcomes¹⁵

As of 2021, 69% of live births in the US are now screened for SMA¹⁵



August 2020

Roche joins the European Alliance for Newborn Screening, led by SMA Europe¹⁶

Following success in US, the European Alliance campaigns for all newborn screening programmes to include a test for SMA by 2025¹⁶

8

August 2020

Roche's molecule is approved in the US for the treatment of SMA in adults and children two months of age and older^{17,18}

9



2020

Throughout the COVID pandemic of 2020, Roche adapts clinical trials to meet the needs of the community

Offering contactless delivery of the treatment to people's homes and home nursing



I am very proud and thankful for everything we have collectively achieved so far and look forward to surmounting the challenges that lie ahead together, and excited to help co-create a better future for people with SMA

Global Head and SVP, Neuroscience and Rare Diseases Clinical Development at Roche

March 2021

Roche's molecule is approved in the European Union for the treatment of SMA in adults and children two months of age and older¹⁹

2021 and beyond

Many challenges remain in SMA, and as our partners and our future look to the future, our unwavering focus remains on working with the community to enable broad treatment access, improve outcomes and build a better tomorrow for all



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