



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...

2011 Roche enters existing collaboration between the SMA Foundation and PTC Therapeutics, with the goal of converting PTC's cutting-



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

Clinical Development²

Neuroscience and Rare Diseases



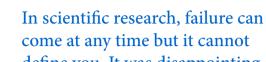
edge technology into a life-saving treatment for people with SMA^{1,2}

molecules with the potential to treat SMA and make a meaningful difference to those living with the condition^{2,3}

2011-2015

((

Scientists from the three partners study several promising



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

2015

Scientists determine that

to be safe and effective, but one promising molecule does emerge³⁻⁶

some investigational molecules are unlikely



promising molecule⁷⁻¹¹

2016

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



 People underrepresented in other SMA studies and from countries not typically included in clinical trials7-11

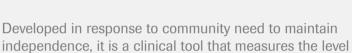
The programme, supported by our partners and designed with the patient and clinical community, includes:

 People with different types of SMA, from birth to 60 years old

 Pre-symptomatic people and those previously treated with other SMA-targeted therapies

with Cure SMA, SMA Europe and people living with SMA¹²

October 2017



SMA Europe

with SMA¹² SMAIS is available to all researchers and healthcare professionals free of charge

of support required to perform smaller daily activities

SMA Independence Scale (SMAIS) developed in collaboration





Created a new means for:

 Promoting patient-centred trial management¹³ SMA Make today a breakthrough.

August 2020

Engaging research sites interested in SMA trials

Providing tangible resources to optimise trial readiness

Newborn Screening, led by SMA Europe¹⁶ Following success in US, the European Alliance campaigns for all newborn screening

Roche joins the European Alliance for

for newborn screening to include an SMA test14 Evidence shows that early diagnosis and treatment of SMA leads to better outcomes15

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2018

Roche joins the US Newborn Screening Coalition to campaign

As of 2021, 69% of live births in the US

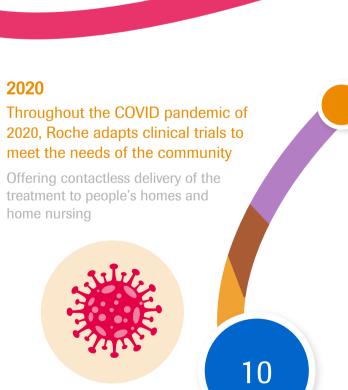
are now screened for SMA15

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

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I am very proud and thankful for everything we have collectively achieved so far and look forward

to surmounting the challenges that

2021 and beyond

Many challenges remain in SMA, and as

Roche and our partners 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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