Roche and Genentech Spinal Muscular Atrophy (SMA) Clinical Development Program

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Roche and Genentech are developing and testing **risdiplam (RG7916), an investigational (unapproved) medicine,** given orally.
According to preclinical data, the investigational medicine, risdiplam, is distributed throughout the body.[1]
Risdiplam is a *SMN2* splicing modifier and is being developed in collaboration with the SMA Foundation and PTC Therapeutics.

Risdiplam*

	2016	2017	2018	2019	2020
	**FIREFISH[2]** Infants (1–7 months) Type 1 SMA	**Part 1: Dose finding complete** Safety and efficacy of risdiplam in the body at different doses[2] N=21[†,3] **Enrollment status: Complete**		**Part 2: Ongoing** Safety and efficacy of risdiplam at the dose selected from Part 1[2] N=41[†,3] **Enrollment status: Complete** **No placebo**	
	**SUNFISH[4]** Children and young adults (2–25 years) Type 2 or 3 SMA	**Part 1: Dose finding complete** Safety and efficacy of risdiplam in the body at different doses[4] N=51[†,5] **Enrollment status: Complete**		**Part 2: Ongoing** Safety and efficacy of risdiplam at the dose selected from Part 1[4] N=180[†,5] **Enrollment status: Complete** **2:1 risdiplam:placebo**	
	**JEWELFISH[6]** Children and adults (6 months–60 years) Non-naïve individuals with SMA		**Ongoing** Safety and tolerability of risdiplam in people who participated in Study BP29420 (MOONFISH) or previously received treatment with nusinersen, olesoxime or onasemnogene abeparvovec-xioi N=174[†,6] **Enrollment status: Complete** **No placebo**		
	**RAINBOWFISH[7]** Infants (Birth–6 weeks) All SMA types		**Ongoing** Safety and efficacy of risdiplam in infants with SMA who are not yet showing symptoms[7] N=25[‡,7] **Enrollment status: Recruiting** **No placebo**		

Current status of the risdiplam clinical study program

FIREFISH

- Infants have received risdiplam for up to 30 months[8]
- Part 2 is ongoing in the USA, Europe, China, Japan, Brazil, Russia, Turkey and Saudi Arabia[2]

SUNFISH

- Participants have received risdiplam for at least 19 months[9]
- Part 2 is ongoing in the USA, Europe, China, Japan, Brazil, Russia, Turkey and Canada[4]

JEWELFISH

- Participants have received risdiplam for up to 29 months[10]
- JEWELFISH is ongoing in the USA and Europe[6]

RAINBOWFISH

- Participants will receive risdiplam daily for 24 months[7]
- RAINBOWFISH is recruiting globally[7]

***This poster contains general information about our SMA program and is not intended as specific medical advice. Risdiplam is an investigational (unapproved) medicine that is being studied for the treatment of people with SMA. Risdiplam has not been approved by the Food and Drug Administration. The efficacy and safety of risdiplam are currently being studied. You should talk with your healthcare provider for information and advice about your condition, including any current or potential treatments.**

[†]Final participant study numbers; [‡]Numbers of participants based on planned enrollment.

References

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