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.

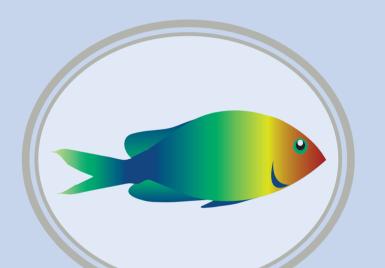
2016	2017	2018	2019	2020
FIREFISH ² Infants (1–7 months)	Part 1: Dose finding complete Safety and efficacy of risdiplam in the body at different doses ² $N=21^{+,3}$		Part 2: Ongoing Safety and efficacy of risdiplam at the dose selected from Part 1 ² N=41 ^{†,3}	
Type 1 SMA	Enrollment status: Complete		Enrollment status: Complete	No placebo



Risdiplam

Children and adults (6 months–60 years) Non-naïve individuals with SMA

participated in Study BP29420 (MOONFISH) or previously received treatment with nusinersen, olesoxime or onasemnogene abeparvovec-xioi $N = 174^{+,6}$ No placebo **Enrollment status: Complete**

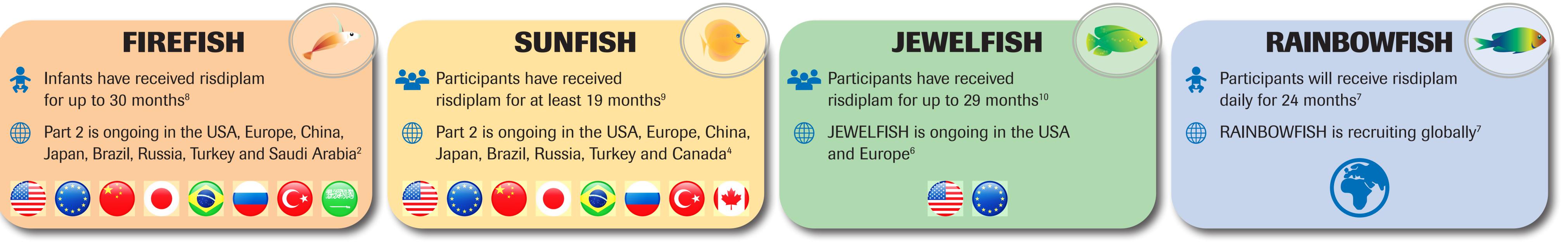


RAINBOWFISH⁷

Infants (Birth–6 weeks) All SMA types

Ongoing Safety and efficacy of risdiplam in infants with SMA who are not yet showing symptoms⁷ N=25^{‡,7}

Current status of the risdiplam clinical study program



*This poster contains general information about our SMA program and 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

Acknowledgments

1. Poirier A, et al. *Pharmacol Res Perspect*. 2018; 29:1–12; 2. Clinicaltrials.gov. NCT02913482 (accessed April 2020); 3. Baranello G, et al. Data presented at CureSMA 2019; 4. Clinicaltrials.gov. NCT02908685 (accessed April 2020);

5. Mercuri E, et al. Data presented at CureSMA 2019; 6. Clinicaltrials.gov. NCT03032172 (accessed April 2020); 7. Clinicaltrials.gov. NCT03779334 (accessed April 2020);

8. Servais L, et al. AAN abstract 2020; 9. Mercuri E, et al. Data presented at WMS 2019;

10. Chiriboga CA, et al. Data presented at WMS 2019.

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