Roche and Genentech Spinal Muscular Atrophy Clinical Development Program

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Roche and Genentech are developing and testing **risdiplam (EVRYSDI™), an oral medicine which has been approved by the Food and Drug Administration (FDA) for the treatment of patients with SMA, aged 2 months and older.**[1] According to preclinical data, risdiplam is distributed throughout the body.[2] 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[3]
Infants
(1–7 months)
Type 1 SMA

Part 1: Dose finding complete
Safety and efficacy of risdiplam in the body at different doses[3]
N=21†,[4]
Enrollment status: Complete

Part 2: Ongoing
Safety and efficacy of risdiplam at the dose selected from Part 1[3]
N=41†,[4]
Enrollment status: Complete
No placebo

SUNFISH[5]
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[5]
N=51†,[6]
Enrollment status: Complete

Part 2: Ongoing
Safety and efficacy of risdiplam at the dose selected from Part 1[5]
N=180†,[6]
Enrollment status: Complete
2:1 risdiplam:placebo

JEWELFISH[7]
Children and adults
(6 months–60 years)
All SMA types

Ongoing
Safety and tolerability of risdiplam in people who previously received treatment with RG7800, nusinersen (SPINRAZA®), olesoxime or onasemnogene abeparvovec-xioi (ZOLGENSMA®)[7]
N=174†,[8]
Enrollment status: Complete
No placebo

RAINBOWFISH[9]
Infants
(Birth–6 weeks)
All SMA types

Ongoing
Safety and efficacy of risdiplam in infants with SMA who are not yet showing symptoms[9]
N=25‡,[9]
Enrollment status: Recruiting
No placebo

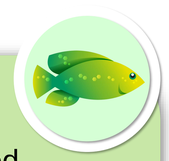
Current status of risdiplam clinical study program

FIREFISH

- Infants have received risdiplam for at least 24 months[10]
- Part 2 is ongoing in the USA, Europe, China, Japan, Brazil, Russia, Turkey and Saudi Arabia[3]

SUNFISH

- Participants have received risdiplam for at least 24 months[11]
- Part 2 is ongoing in the USA, Europe, China, Japan, Brazil, Russia, Turkey and Canada[5]



JEWELFISH

- Participants have received risdiplam for up to 32.8 months[8§]
- JEWELFISH is ongoing in the USA and Europe[7]

RAINBOWFISH

- Participants will receive risdiplam daily for 24 months[12]
- RAINBOWFISH is recruiting globally[9]

***This poster contains general information about our SMA program and is not intended as specific medical advice and should not be construed as a recommendation for the use of any product for unapproved uses. Risdiplam has only been approved by the US FDA for the treatment of patients with SMA, aged 2 months and older. This compound and its use may not be approved in your country. 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. §Data cut off: 31 Jan 2020.

References

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Acknowledgments

We thank our collaborators and participants who take part in our clinical trials. The logos are the property of the companies listed. Editorial support was provided by Lauren Walmsley, PhD, of MediTech Media UK, funded by F. Hoffmann-La Roche Ltd. Genentech is a member of the Roche group.

M-XX-00001681

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