MEDIA RELEASE

Roche's Evrysdi® (risdiplam) – the first and only oral treatment for adults and children two months or older suffering from Spinal Muscular Atrophy (SMA), now available in India

- This disease modifying therapy is the first and only approved treatment available in India for SMA patients across all types.
- Evrysdi® is being studied as part of the broadest and largest global clinical trial program in SMA - from newborn infants to adults aged 60 years, with varying symptoms and motor function.

29 July 2021, Mumbai, India: Roche today announced the launch of Evrysdi® (risdiplam), the first and only approved treatment in India for Spinal Muscular Atrophy (SMA) patients. Evrysdi® was first approved by the US FDA in August 2020 and is today available in India within 11 months of the US approval. Since its launch, over 4000 SMA patients across 50+ countries have benefitted from Evrysdi®.

About SMA

SMA is a severe, progressive rare neuromuscular disease that can be fatal. It affects approximately one in 10,000 live births globally[1] and one in 7744 live births in India[2] and is the leading genetic cause of infant mortality. SMA is caused by a mutation of the survival motor neuron 1 (*SMN1*) gene, which leads to a deficiency of SMN protein. This protein is found throughout the body and is essential to the function of nerves that control muscles and movement. Without it, nerve cells cannot function correctly, leading to muscle weakness over time. Depending on the type of SMA, an individual's physical strength and their ability to walk, eat or breathe can be significantly diminished or lost.

About Evrysdi® and global clinical trials

Evrysdi® is administered daily at home orally (it is supplied as powder which is constituted into a liquid solution and taken once daily by mouth or feeding tube if required) and is designed to treat SMA by increasing production of the Survival Motor Neuron (SMN) protein. It is approved for the treatment of spinal muscular atrophy (SMA) in adults and children 2 months of age and older.

Evrysdi® is being studied in more than 450 people as part of the broadest, large and robust clinical trial program in SMA. The program included newborn infants to adults aged 60 years with varying symptoms and motor function, and is the only program that has included those that were previously treated for SMA with another medication.
Evrysdi® was approved by Indian Health Authorities after reviewing its efficacy and safety data from three global clinical studies designed to represent a broad spectrum of people living with SMA:

1. **FIREFISH**[3] in symptomatic infants aged 1 to 7 months with Type 1 SMA
2. **SUNFISH**[3] in children and adults aged 2 to 25 years. SUNFISH is the first and only placebo-controlled trial to include adults with Types 2 and 3 SMA.

Evrysdi® showed clinically-meaningful improvements in motor function across these two clinical trials in people with varying ages and levels of disease severity, including Types 1, 2, and 3 SMA. Infants achieved the ability to sit without support for at least 5 seconds, a key motor milestone not

normally seen in the natural course of the disease. Evrysdi® also improved survival without permanent ventilation at 12 and 23 months, compared to natural history. Evrysdi maintained the ability to swallow and feed orally in the majority of the infants after 2 years of therapy. This is a unique characteristic of Evrysdi that was not observed with other medications for SMA.

3. **JEWELFISH**[3] (interim data) is a study assessing people with any SMA type and disease severity who have previously been treated with other SMA targeted therapies between 6 months and 60 years. This study enrolled the broadest patient population ever studied in an SMA trial. Data from the JEWELFISH study, which included a diverse patient population with a high degree of motor impairment, show that Evrysdi® has a favorable safety profile in patients previously treated with other SMA targeting therapies. Evrysdi also showed to improve SMN protein levels to 2 fold and above in all the patients who have received a previous therapy.

Evrysdi's clinical development program also includes:
RAINBOWFISH[3] is a study assessing pre-symptomatic infants with Type 1 SMA from birth to 6 weeks old. Preliminary efficacy data from RAINBOWFISH showed that infants treated for 12 months achieved age appropriate motor milestones within the WHO windows for healthy children, including sitting, standing and walking, and improvements in motor function.

V Simpson Emmanuel, CEO and Managing Director, Roche Pharma India says, "The launch of Evrysdi® in India is a fine example of Roche living its purpose of 'Doing now what patients need next'. We are betting big on solving complex challenges related to rare diseases as we believe no patient should be deprived an opportunity to live a healthy life, however complex or rare the disease is. Today heralds a new journey of hope as we are all coming together to add color into the lives of SMA patients in India with the launch of Evrysdi®, the first and only approved treatment in India for patients living with SMA. This also marks our foray into rare disease treatment in India, he added."

Patient Support Program (PSP) to drive access

To ensure every patient gets access to this disease modifying therapy, Roche is announcing its Patient Support program (PSP) for Evrysdi®. Through this program:

- In the first two years of treatment, Roche provides three bottles free for every two bottles bought by the patient
- From the third year onwards, Roche provides two bottles free for every one bottle bought by the patient

To provide a holistic solution to SMA patients and caregivers, we identified the most important challenges they face throughout their journey and have designed solutions to support them to mitigate those challenges. Through our PSP program, Roche will provide services like: Physiotherapy, Financial counselling, Psychosocial and Nutritional counselling to SMA patients.

Patients and caregivers can reach out to us at **1800-202-4755** for information on the PSP program.

Home delivery of Evrysdi®: SMA patients have weak pulmonary health and find it difficult to visit a hospital to receive therapy, especially in COVID times. Moreover, motor disability adds to this burden and makes it very difficult for these patients and caregivers to travel to receive the drug. To solve this challenge, Roche will provide free home delivery of Evrysdi® to each and every patient following consent

from the patient/ caregiver and their HCPs. Being the only oral medication for SMA that can be administered in a home setting, this facility will add tremendous convenience to both patients and their caregivers, especially the added challenge Covid-19 poses. Patients, caregivers and HCPs can reach out to us at **1800-202-4755** to know more about Evrysdi's availability and free home delivery.

"Given the majority of people with SMA in India remain untreated, we believe Evrysdi, with its highly efficacious clinical profile and oral administration advantage, will offer meaningful benefits for many living with this rare neurological disease," said **Dr. Bruno Jolain, Chief Medical Officer, Roche Pharma India**. "Administration of Evrysdi requires no hospitalization, no anesthesia, no specialized care center, no complex administration and no steroids. A simple oral administration gives SMA patients, treating physicians and caregivers more control over their daily lives."

"Throughout their lives, many people with SMA may lose their ability to perform critical movements, which can impact the ability to independently participate in different aspects of daily life and even be life altering. SMA patients in India have long lived without a viable treatment option. We are today encouraged by the availability of an approved solution in India," said **Alpana Sharma, Co-Founder & Director Patient Advocacy at CureSMA Foundation of India.**

"The launch of Evrysdi in India is an eagerly awaited milestone for our community. We appreciate Roche's commitment in making this drug available in India soon after its global launch and in developing a treatment that can be administered at home," said **Archana Vashist Panda, Co-Founder & Director Patient Advocacy, CureSMA Foundation of India.**

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[1] *https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5502065/*
[2] *https://onlinelibrary.wiley.com/doi/abs/10.1111/cge.13796*
[3] *Additional details regarding the clinical trials are available in the annexure: Evrysdi: Summary of Key Facts*

About Roche Products (India) Pvt. Ltd.

Roche Products (India) Private Limited was incorporated in 1994 as a wholly owned subsidiary of the Roche Group, headquartered in Basel, Switzerland. Roche is the world's largest biotech company, with truly differentiated medicines in oncology, immunology, infectious diseases, ophthalmology and diseases of the central nervous system. For more than 60 years, Roche has been committed to making a difference to the lives of people in India. Today, Roche is the leader in oncology treatment in India; apart from cancer, Roche's has innovative medicines in other therapy areas too: transplantation, rheumatoid arthritis (RA), and chronic kidney disease (CKD)-related anaemia. Roche believes in making the latest and most innovative medicines accessible to patients in India in the fastest possible time. For more

than 50 years, Roche has been developing medicines with the goal to redefine treatment in oncology. Today, Roche is investing more than ever in our effort to bring innovative treatment options that help a person's own immune system fight cancer. For more information on Roche Pharma India, visit www.rocheindia.com.

For further information, please contact:

ROCHE:

S. Rajan
Chief Corporate Affairs and Communications Officer
Mobile: +91 9930109372| Email: rajan.s@roche.com

Christina Dsouza
SPAG
Mobile: +91 97690 27774| Email: christina.dsouza@spag.asia