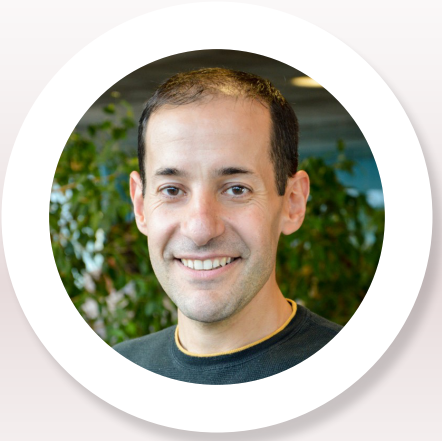
Hasane Ratni, Med Chem 2019, Volume 09
DOI: 10.4172/2161-0444-C1-047

11th International Conference on
Medicinal Chemistry & Pharmaceutical Technology
April 01-02, 2019 | Prague, Czech Republic

Discovery of risdiplam, a selective survival of motor neuron-2 (SMN2) gene splicing modifier for the treatment of spinal muscular atrophy (SMA)



Hasane Ratni
F. Hoffmann-La Roche Ltd.,
Switzerland

SMA is an inherited disease that leads to loss of motor function and ambulation, and a reduced life expectancy. We have been working to develop orally-administrated, systemically-distributed small molecules to increase levels of functional SMN protein. Our initial development candidate RG7800 was the first SMN2 splicing modifier tested in clinical trials in healthy volunteers and SMA patients. It was safe and well tolerated, and increased SMN protein levels up to two fold in patients. Nevertheless, its development was stopped as a precautionary measure because retinal toxicity was observed in cynomolgus monkeys after chronic daily oral dosing (39 weeks), at exposures in large excess of those investigated in patients. Herein, we describe the

Dis**COV**ery risdiplam (RG7916, RO7034067) that focused on thorough pharmacology, DMPK and safety characterization and optimization. This compound is undergoing pivotal clinical trials and is a promising medicine for the treatment of patients in all ages and stages with SMA.

Biography

Hasane Ratni is an Expert Scientist in Medicinal Chemistry at F. Hoffmann-La Roche Ltd., pRED, Pharma Research and Early Development, Roche Innovation Center Basel, Switzerland. He received his PhD at the University of Geneva. His research has mainly been devoted to the areas of Neuroscience (for example neurokinin receptors, or V1a receptor antagonist now in late stage human clinical trials for autism). In 2005, he participated in a secondment within the Roche group at Chugai Pharmaceutical Co. Ltd, Gotemba Japan, in the field of renal disease. He was the Chemistry Discovery Project Leader of the SMN program aiming for a treatment for spinal muscular atrophy, now completing pivotal clinical trials in patients. His current focus is on gamma secretase modulator for Alzheimer's disease. He is an Author and Co-author of more than 110 patents and publications. In 2014, he received the Roche Leo Sternbach Award for Innovation in Chemistry and Gold medal at the Roche Patent Inventor's recognition event in 2016.

hasane.ratni@roche.com

Notes: