

## Prosena explain Exon Skipping in Duchenne Muscular Dystrophy at 6th annual RNA Therapeutics Summit

RNA Therapeutics, 16th & 17th February, London UK

LONDON, LONDON, UNITED KINGDOM, November 27, 2014 /EINPresswire.com/ -- It was announced earlier this week that US Drug maker BioMarin will acquire biopharmaceutical company Prosensa, for a total of \$680 million. The deal includes two additional payments of roughly \$80 million, tied to regulatory approvals of a Duchenne drug currently being developed by Prosensa.

Prosensa is one of a few companies developing treatments for the progressive muscle-wasting disorder, Duchenne and is advancing an RNA-based treatment that targets an abnormality in part of the dystrophin gene. Both the Prosensa and Sarepta Therapeutics target

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abnormalities in exon 51, which affects 13 percent of patients with Duchenne.\*

Hear more from both Prosensa and Serepta Therapeutics at SMi's 6th annual conference on RNA Therapeutics, when it returns to London on 16th & 17th February 2015.

Prosensa Chief Medical Officer and SVP of R&D, Giles Campion, will be providing attendees with unique insights in a presentation entitled: Exon Skipping in Duchenne Muscular Dystrophy. The address will deliver learnings from a global clinical programme, discuss splice switching and offer guidance on accelerating regulatory paths to approval. He will then go onto leading a round table discussion on developing RNA Therapeutics for rare diseases.

A keynote address from Sarepta entitled: From RNA splicing to Eterplirsen trials - a drug for Duchenne muscular dystrophy, will be presented by Distinguished Scientist, Ryszard Kole. Sarepta Therapeutics will present their findings from a 120 day week trial, discuss the invention of RNA splicing and provide insight into Eteplirsen induced exon skipping of 51 in dystrophin premRNA in DMD patients.

Further details are available online at <a href="https://www.therapeutics-rna.com">www.therapeutics-rna.com</a>

RNA Therapeutics: From Cell to Sales – Clearing the Path to Market 16-17 February 2015

Marriott Regents Park Hotel, London UK

www.therapeutics-rna.com

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Source: <a href="http://www.xconomy.com/san-francisco/2014/11/24/biomarin-gambles-840m-on-prosensas-duchenne-drug/">http://www.xconomy.com/san-francisco/2014/11/24/biomarin-gambles-840m-on-prosensas-duchenne-drug/</a>

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