Exicure to Present Data at the Cure Spinal Muscular Atrophy Annual Conference in Dallas

June 14, 2018

Exicure's three-dimensional spherical nucleic acid containing the nusinersen sequence prolongs survival and reduces toxicity compared to nusinersen in a spinal muscular atrophy mouse model

SKOKIE, Ill.--(BUSINESS WIRE)--Jun. 14, 2018-- Exicure, Inc. (OTCQB:XCUR), the pioneer in gene regulatory and immunotherapeutic drugs utilizing three-dimensional, spherical nucleic acid (SNA™) constructs, announced today that Exicure and its collaborators at The Ohio State University Wexner Medical Center will show preclinical data demonstrating the performance of Exicure's SNA compound designed for use in spinal muscular atrophy (SMA). These data will be presented at the Cure SMA Annual Conference in Dallas, Texas on June 14, 2018.

"Exicure's spherical nucleic acid version of nusinersen demonstrates increased survival and decreased toxicity in the translationally-relevant SMA mouse model," said David Giljohann, PhD, CEO of Exicure. "We believe these results are important for developing improved treatments for patients with SMA. These data also suggest that Exicure's technology platform could potentially create more potent therapies for other disorders of the central nervous system, including Huntington's disease, Alzheimer's disease, and Parkinson's disease."

At the meeting, Arthur Burghes, PhD, from Ohio State's Wexner Medical Center, and Exicure will present data from preclinical studies in a SMA mouse model. The poster is titled "Nusinersen in spherical nucleic acid (SNA) format improves efficacy both *in vitro* in SMA patient fibroblasts and in Δ7 SMA mice and reduces toxicity in mice." The presentation will highlight that Exicure's proprietary technology:

- Prolonged survival by four-fold (maximal survival of 115 days compared to 28 days for nusinersen-treated mice);
- Doubled the levels of healthy full-length SMN2 mRNA and protein in SMA patient fibroblasts when compared to nusinersen;
- Doubled the quantity of healthy full-length SMN mRNA levels in spinal cord tissue compared to untreated mice;
- Mitigated toxicity of nusinersen at the highest dose tested in mice.

In August 2017, Exicure and The Ohio State University established a collaboration to further validate and characterize the pharmacology of Exicure's nusinersen-SNA compound in mouse models. This collaboration's ongoing *in vivo* research is conducted by Dr. Burghes, an internationally known researcher, leading basic and clinical research on SMA and other genetic neuromuscular disorders.

About Exicure, Inc.

Exicure, Inc. is a clinical stage biotechnology company developing a new class of immunomodulatory and gene regulating drugs against validated targets. Exicure's proprietary 3-dimensional, spherical nucleic acid (SNA™) architecture unlocks the potential of therapeutic oligonucleotides in a wide range of cells and tissues. Exicure's lead programs address oncology, inflammatory diseases and genetic disorders. Exicure is based outside of Chicago, IL. For more information, please visit www.exicuretx.com.

About Spinal Muscular Atrophy (SMA)

SMA is the most common genetic cause of death for infants. SMA results from the loss of the SMN1 gene and an inability of SMN2 to produce sufficient full-length protein to make up for the loss of SMN1. The SMN1 gene, in a healthy person, produces a full-length protein that is essential to the function of the nerves that control muscles. Without sufficient SMN protein, the nerve cells cannot properly function and eventually die. This leads to debilitating and even fatal muscle weakness.

About Nusinersen

Nusinersen, marketed as Spinraza® by Biogen, is a modified antisense oligonucleotide. In December of 2016, nusinersen was approved by the US FDA for the treatment of SMA in pediatric and adult patients.

Forward Looking Statements

This press release contains forward-looking statements (including within the meaning of Section 21E of the United States Securities Exchange Act of 1934, as amended, and Section 27A of the United States Securities Act of 1933, as amended) concerning the Company, the Company's technology, potential therapies, pre-clinical results, and other matters. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer to future events or conditions, and include words such as "may," "will," "should," "would," "expect," "plan," "believe," "intend," "look forward," and other similar expressions among others. Statements that are not historical facts are forward-looking statements. Forward-looking statements are based on current beliefs and assumptions that are subject to risks and uncertainties and are not guarantees of future performance. Actual results could differ materially from those contained in any forward-looking statement as a result of various factors, including, without limitation: that Exicure's pre-clinical programs do not advance into the clinic or result in approved products on a timely or cost effective basis or at all; regulatory developments; and the ability of Exicure to obtain sufficient funding for its programs and to protect its intellectual property rights. Exicure's pipeline programs are in various stages of pre-clinical and clinical development, and the process by which such pre-clinical or clinical therapeutic candidates could potentially lead to an approved therapeutic is long and subject to significant risks and uncertainties. Risks facing the Company and its programs are set forth in the Company's filings with the SEC. Except as required by applicable law, the Company undertakes no obligation to revise or update any forward-looking statement, or to make any other forward-looking statements, whether as a result of new information, future events or otherwise.

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