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Drug Discovery of Therapies for Duchenne Muscular Dystrophy

CHICAGO – A new review article published in the December 2015 issue of the *Journal of Biomolecular Screening* (JBS) details state-of-the-art drug discovery research to identify therapies for Duchenne Muscular Dystrophy (DMD). DMD is a devastating, incurable, genetic muscle-wasting disorder affecting one of every 3500-5000 males born. The review is authored by Yuval Blat, Ph.D., formerly the director of biology at Armgo Pharma, a company developing drugs for DMD; and Shachar Blat of the Department of Neuroscience, University of Pittsburgh.

With increased industry interest in targeting rare diseases, novel DMD therapies are being explored by a large number of companies and academic institutes. The review describes the most promising directions, covering all existing treatment modalities, such as small molecules, proteins and RNA.

The review also provides a comprehensive summary of all pathways that are believed to be involved in the pathophysiology of DMD and could serve as potential entry points for a therapeutic intervention. Among these are alterations in calcium homeostasis, oxidative stress, inflammation, fibrosis and protease activation.

In order to facilitate future drug discovery efforts for DMD, the review details the tools currently available to researchers. In vitro tools include myoblast-like cell lines for high-throughput compound screening as well as primary muscle fibers and cardiomyocytes for in-depth mechanistic studies. Genetic animal models such as skeletal muscle and cardiac functional readouts in the mdx mouse model allow for in vivo compound testing in context relevant to the human disease. Finally, the strategies and outcome measures followed in current DMD clinical trials are discussed.

JBS is one of two MEDLINE-indexed scientific journals published by the Society for Laboratory Automation and Screening (SLAS). Visit JBS Online at <http://jbx.sagepub.com/content/20/10> to read “Drug Discovery of Therapies for Duchenne Muscular Dystrophy.” For more information about SLAS and its journals, visit www.slas.org/jala-jbs.

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The Society for Laboratory Automation and Screening (SLAS) is an international community of more than 15,000 individual scientists, engineers, researchers, technologists and others from academic, government and commercial laboratories. The SLAS mission is to be the preeminent global organization providing forums for

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education and information exchange and to encourage the study of, and improve the practice of laboratory science and technology. For more information, visit www.SLAS.org.

SLAS publishes two internationally recognized, MEDLINE-indexed journals, now in their 20th year of publication. **The Journal of Laboratory Automation (JALA)** and **Journal of Biomolecular Screening (JBS)** uniquely serve laboratory science and technology professionals who work primarily in life science R&D. Together, JALA and JBS address the full spectrum of issues that are mission-critical to this important audience, enabling scientific research teams to gain scientific insights, increase productivity, elevate data quality, reduce lab process cycle times and enable experimentation that otherwise would be impossible.

Specifically, **JALA** explores ways in which scientists adapt advancements in technology for scientific exploration and experimentation. In direct relation to this, **JBS** reports how scientists use adapted technology to pursue new therapeutics for unmet medical needs, including assay development, identification of chemical probes and target identification and validation in general.

Journal of Biomolecular Screening (JBS): 2013 Impact Factor 2.423. Editor-in-Chief Robert M. Campbell, Ph.D., Eli Lilly and Company, Indianapolis, IN (USA).

Journal of Laboratory Automation (JALA): 2013 Impact Factor 1.879. Editor-in-Chief Edward Kai-Hua Chow, Ph.D., National University of Singapore (Singapore).