Contacts:
Jim Brown
MDA Vice President – Public Relations
520-529-5317 (o); 520-903-8556 (c); jbrown@mdausa.org

Dr. Joel B. Braunstein
Co-Founder & CEO, Tivorsan Pharmaceuticals
410-653-9685; jbraun@tivorsan.com

MDA AWARDS $1 MILLION TO TIVORSAN PHARMACEUTICALS FOR ACCELERATING PIVOTAL PRE-CLINICAL WORK ON TVN-102 AS A POTENTIAL MUSCULAR DYSTROPHY TREATMENT

TUCSON, Ariz. and PROVIDENCE, R.I., Jan. 5, 2012 – The Muscular Dystrophy Association (MDA) today announced it has awarded $1.0 million to Tivorsan Pharmaceuticals to help speed pre-clinical work vital to a filing of an Investigational New Drug (IND) application with the U.S. Food and Drug Administration (FDA) for a recombinant humanized form of biglycan as a potential muscular dystrophy treatment. The new funding to the Providence-based biotechnology firm comes from MDA Venture Philanthropy (MVP), a part of MDA's translational research program.

The project being advanced by Tivorsan is focused on treating Duchenne muscular dystrophy (DMD) by up-regulating the utrophin protein that's been shown to provide some compensation for disease-causing dystrophin deficiency in animals with DMD-like muscular dystrophy. The company's TVN-102 experimental drug is based on biglycan, a naturally occurring protein found on the membrane surrounding each muscle fiber. In the standard mouse model of DMD, biglycan reduced muscle damage, reduced muscle degeneration and improved muscle function.

DMD is one of nine types of muscular dystrophy, a group of genetic, degenerative diseases primarily affecting voluntary muscles. Caused by mutations in the gene that makes dystrophin, a protein that normally protects muscle cells and keeps them intact, DMD eventually weakens all voluntary muscles, and the heart and breathing muscles. DMD affects 1 in 3,500 boys with an estimated patient population exceeding 50,000 worldwide.

"We are delighted to have a productive collaboration with Dr. Justin Fallon's laboratory at Brown University to bring such a promising potential muscular dystrophy treatment to market," said Joel B. Braunstein, M.D., co-founder and CEO of Tivorsan Pharmaceuticals. "The exciting progress we

are making would not be possible without strong support from the National Institutes of Health (NIH), MDA and other industry partners."

MDA, for example, has played a pioneering role in utrophin-related research since 1996 (investing nearly $16 million to help develop this promising therapeutic pathway). MDA also was an early sponsor of Dr. Fallon's insightful research on muscle cell biology (1994-2000) that ultimately led to the 2010 discovery that Biglycan could serve as a molecular magnet to attract the utrophin protein.

Fallon and others found that giving systemic injections of recombinant biglycan to dystrophin-deficient mice caused utrophin to accumulate at the right position near the muscle cell membrane, reducing structural muscle abnormalities and improving muscle function.

Animal efficacy studies also have shown that systemically administered recombinant biglycan is well-tolerated and remains effective for approximately three weeks following a one-time dosing. Therapeutic effects persist out to three months following dosing once every three weeks.

"MDA is excited about enabling Tivorsan to complete vital pre-clinical activities," said Valerie Cwik, M.D. Executive Vice President Research and Medical Director for the Muscular Dystrophy Association. "MDA has long been a champion for utrophin upregulation as a promising therapeutic strategy for treating Duchenne muscular dystrophy. Tivorsan's biglycan drug has the potential to effectively attract the utrophin protein to where it could have a clinically beneficial effect in muscle cells."

About Utrophin

Utrophin is a naturally-occurring protein that has a similar function to dystrophin. Utrophin is produced during fetal development but its production is switched off in adults. If utrophin production could be maintained, it could act as a substitute for the missing dystrophin to maintain the healthy function of muscles. One method of turning utrophin production back on is through pharmacological means. Utrophin upregulation has the potential to be beneficial to all DMD patients regardless of their specific genetic mutation. It is also expected to be complementary to other therapeutic approaches in development.

About Muscular Dystrophy Association

MDA is the nonprofit agency dedicated to curing muscular dystrophy, amyotrophic lateral sclerosis (ALS) and related diseases by funding worldwide research. The Association also provides comprehensive healthcare and support services, advocacy and education.

Long the world's leading non-governmental funder of muscular dystrophy research and health care services to affected individuals, MDA's active grants include 102 focused on Duchenne muscular dystrophy, representing a multi-year investment of close to $35 million.

In addition to annually funding some 300 research teams worldwide, MDA maintains a national network of some 200 hospital-affiliated clinics; facilitates hundreds of support groups for families affected by neuromuscular diseases; and provides extraordinary local summer camp opportunities for thousands of youngsters fighting progressive muscle diseases. Known globally for its Labor Day Telethon, MDA is the first nonprofit to receive a Lifetime Achievement Award from the American Medical Association "for significant and lasting contributions to the health and welfare of humanity." To learn more, watch the award-winning "Make a Muscle, Make a Difference PSA, visit mda.org or follow MDA on FaceBook or Twitter.

About Tivorsan Pharmaceuticals

Tivorsan Pharmaceuticals is a protein therapeutics company pioneering a unique approach to treating serious neuromuscular disorders, including DMD and Becker Muscular Dystrophy (BMD).

This method, using recombinant human biglycan (rhBGN), is based on 25 years of basic science work in the Fallon laboratory at Brown University. Tivorsan was formed by Dr. Justin Fallon in collaboration with colleagues from Old Forge Holdings, LLC of Greenwich, CT and LifeTech Research, Inc, a Baltimore, MD-based technology research and development firm.