

# VASTox plc ("VASTox" or "the Company")

## VASTOX SELECTS PRECLINICAL CANDIDATE IN DUCHENNE MUSCULAR DYSTROPHY PROGRAMME

**Oxford, UK, 8 May 2007** – VASTox plc (AIM: VOX), a leading UK biotechnology company, announces that it has selected a candidate to enter into preclinical development in the Company's Duchenne Muscular Dystrophy ("DMD") drug discovery programme.

VASTox has a unique therapeutic approach that targets the underlying cause of this disease. Owing to a genetic defect, DMD patients lack an important protein called dystrophin, which results in severe muscle wasting and is ultimately fatal for patients. VASTox is developing a small molecule treatment that replaces the missing dystrophin by increasing levels of a functionally similar protein called utrophin.

A significant benefit of VASTox's approach of 'up-regulating' utrophin is that it should be effective in treating all DMD patients and, importantly, the Company expects any drug it develops to be complementary with current palliative treatments and the variety of other scientific approaches currently aimed at discovering effective DMD therapies.

VASTox's preclinical candidate, called VOX C1100, was selected from a series of promising compounds after it significantly reduced levels of muscle degeneration during *in vivo* studies. Other key secondary symptoms of DMD relating to the chronic inflammation and fibrosis of muscles also showed significant improvement in the same investigations. Higher levels of utrophin were identified in treated muscles with these data providing validation of VASTox's unique approach for developing a treatment for DMD.

The novel candidate, VOX C1100, has advantageous chemical and pharmaceutical properties suitable for a small molecule oral drug. Furthermore, it has successfully completed preliminary safety and toxicity testing after being screened through VASTox's zebrafish chemical genomics platform. VASTox expects to submit an Investigational New Drug ("IND") filing for VOX C1100 by mid 2008 with the compound entering first-in-man Phase I clinical trials during the second half of 2008.

Orphan drug designation has been granted for utrophin up-regulation as a mechanism of treating DMD by the European Medicines Agency (EMEA) and this status will provide VASTox with considerable support during drug development by accelerating this process and reducing the associated costs. The Company has also built up a strong patent estate to protect the increasing value of this drug discovery programme with several patents, either granted or filed, covering all of the World's major markets.

Orphan disease indications are rare diseases with relatively small patient populations but are commercially attractive with current marketed orphan drugs generating average annual sales in excess of \$500 million. VASTox is actively pursuing commercial partnering collaborations for the Company's increasingly valuable DMD preclinical programme. Richard Storer, DPhil, VASTox's Chief Scientific Officer, commented: "The selection of this candidate for development is the culmination of over two years of dedicated efforts by our research teams. It is particularly gratifying that a compound initially identified as a result of *in vitro* utrophin up-regulation produces the benefits observed *in vivo* and thereby supports our fundamental scientific approach."

Steven Lee, PhD, CEO of VASTox added "The selection of VOX C1100 represents the achievement of a major milestone for our DMD programme and illustrates the excellent progress this programme has made over the past two years. With the progression of DMD into preclinical development, VASTox now has five programmes across a range of therapeutic areas that are either in clinical or preclinical development and indicates the strength, depth and ever increasing value of our discovery pipeline."

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### For more information please contact:

VASTox Steven Lee, PhD, Chief Executive Officer Richard Storer, DPhil, Chief Scientific Officer	Tel: +44 (0)1235 443951
Citigate Dewe Rogerson Mark Swallow / David Dible / Valerie Auffray	Tel: +44 (0)207 638 9571

# About VASTox's DMD programme

DMD is a devastating disease that affects young males for which there is currently no effective treatment. Patients rarely survive beyond the age of 25 years.

VASTox has demonstrated *in vivo* up-regulation (increased production) of the protein utrophin by a number of small molecules from their proprietary chemical library. This is a significant development as utrophin has been demonstrated to replace the function of dystrophin, which is missing in DMD patients. Up-regulation of utrophin is widely viewed by the scientific community as a highly promising approach for the development of an effective treatment for DMD.

# About VASTox plc

VASTox is a leading UK biotechnology company that discovers and develops proprietary new drugs. The Company's internal drug development programmes are underpinned by its advanced chemistry and drug screening (chemical genomics) technology platforms, which it also provides on a collaborative or fee-for-service basis to the pharmaceutical industry.

VASTox has a broad range of drug discovery programmes in the clinical, pre-clinical and discovery stages of development, which target serious diseases with a high unmet medical need. These therapeutic areas include neuro-disorders (neurodegenerative and neuromuscular), anti-infectives, ophthalmic diseases, oncology and regenerative medicines.

VASTox's in-house drug development capabilities combine world-class expertise in both medicinal and carbohydrate chemistry with high-volume, high-content screening

using its proprietary zebrafish and fruitfly technologies (chemical genomics). These whole organism screens have the potential to dramatically decrease the time and cost of drug discovery and development by delivering data that are highly predictive of the efficacy and toxicity of potential drug compounds in humans.

The company listed on the AIM market of the London Stock Exchange in October 2004 – symbol: VOX

Further information about the company is available at www.vastox.com

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Forward-looking statements are based on the Company's current expectations and assumptions regarding our business, the economy and other future conditions. Because forward-looking statements relate to the future, by their nature, they are subject to inherent uncertainties, risks and changes in circumstances that are difficult to predict. The Company's actual results may differ materially from those contemplated by the forward-looking statements. The Company cautions you therefore that you should not rely on any of these forward-looking statements as statements of historical fact or as guarantees or assurances of future performance. Important factors that could cause actual results to differ materially from those in the forward-looking statements and regional, national, global political, economic, business, competitive, market and regulatory conditions.