

LBA2

A PHASE 2 STUDY - TENGION AUTOLOGOUS NEO-BLADDER AUGMENT™ (NBA) FOR AUGMENTATION CYSTOPLASTY IN SUBJECTS WITH NEUROGENIC BLADDER SECONDARY TO SPINA BIFIDA

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INTRODUCTION AND OBJECTIVE: The ability to use an autologous cell-seeded biodegradable scaffold for bladder augmentation was demonstrated in patients with neurogenic bladder (NB) due to spina bifida (SB) at Children's Hospital Boston (CHB) [1]. We conducted a confirmatory prospective multicenter Phase 2 study of the Tengion NBA in a similar population.

METHODS: Male or female subjects, 3 to 21 years, with NB due to SB, were eligible if they required augmentation cystoplasty for bladder pressure >40cmH₂O and/or new onset of upper tract changes. Eligibility was confirmed by a Steering Committee. Bladder neck sling was the only concomitant surgical procedure permitted. Since biomechanical stimulation (cycling) promotes tissue regeneration, patients were required to bladder cycle postoperatively. Following an open bladder biopsy, urothelial and smooth muscle cells were grown ex vivo for 5 - 7 weeks, then seeded onto a biodegradable scaffold (the NBA). The implanted NBA served as a template for bladder tissue regeneration. The primary endpoint was urodynamic (UDS) compliance 1 year post implantation. Evaluations included cystograms, renal ultrasounds, physicals and labs.

RESULTS: Four centers enrolled 11 subjects; 10 (6 females) were implanted. Mean age was 8.2 [3-16] years. The procedure was generally well tolerated. Six patients able to bladder cycle showed clinical improvement. Overall, UDS changes were consistent with those from CHB. Hydronephrosis and/or reflux improved/resolved in 4/5 patients. Patients unable to cycle (3 concomitant bladder neck slings, 1 low pressure high grade reflux) showed no UDS improvement at 12 months.

CONCLUSIONS: The study supports the potential of regenerative medicine in bladder augmentation. Long term follow-up is ongoing. Additional studies are needed to confirm the benefits of this promising technology.

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