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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Table 1

<table>
<thead>
<tr>
<th>Procedure</th>
<th>Description</th>
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<tbody>
<tr>
<td>Open biopsy</td>
<td>Biopsy of bladder for procurement of autologous bladder cells</td>
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<td>Cells seeded</td>
<td>Seeded onto a biodegradable scaffold for implantation</td>
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<td>Surgical attachment</td>
<td>Attachment of the NBA to the dome of the native bladder</td>
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<td>Vascularization</td>
<td>Enhanced through mobilization and wrapping of omentum around the NBA</td>
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<td>Bladder neck sling</td>
<td>Sling was performed concomitantly if deemed necessary during screening</td>
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<td>Subjects required</td>
<td>Subjects were required to cycle intermittently fill and empty their bladder</td>
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<td>Figure 1</td>
<td><img src="image1.png" alt="Implant of NBA at dome of native bladder" /></td>
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<tr>
<td>Figure 2</td>
<td><img src="image2.png" alt="Wrapping of omentum around NBA for vasocellularization" /></td>
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</table>

RESULTS OF NON-RESPONDERS:
- 4 patients had at least one concomitant anatomic abnormality that could interfere with their ability to cycle effectively:
  - 1 patient had severe low pressure, high grade reflux, impeding her ability to retain urine in her bladder in order to cycle
  - 3 patients had open bladder necks (all underwent bladder neck slings at NBA implantation)
- Incontinence improved only in the 1 patient undergoing bladder neck closure. No change was reported for the other 3 patients

RESULTS OF NON-RESPONDERS:
- 6 patients showed UDS and/or clinical improvement (Figure 3)
- Per patient/parent-reported assessment, continence improved in 5/5 with baseline incontinence
  - 1 with baseline continence: stable
  - Hydronephrosis and/or reflux improved/resolved in 4/5 patients

RESULTS OF NON-RESPONDERS:
- Incontinence improved only in the 1 patient undergoing bladder neck closure. No change was reported for the other 3 patients
- Hydronephrosis: improved in 1 patient with baseline hydronephrosis
- VUR: unchanged in the 2 patients with baseline VUR

SAFETY:
- Table 2: Treatment Emergent Adverse Events occurring in ≥5 patients: during or within 30 days post-implantation

IV. CONCLUSIONS:
- Feasibility of Tengion NBA demonstrated by improved UDS, continence, radiographics, and clinical assessments
- This study replicates the results of the original neo-bladder augment reported by investigators from CHB
- Cycling is an important determinant for successful regeneration
- Patients with anatomic barriers to bladder cycling (e.g., open bladder necks) may have challenges with successful bladder regeneration
- Urodynamic Endpoint: Compliance at cystometric capacity as a single endpoint is not reflective of the success or failure of this technology
- Evaluation of the Pressure-Volume relationship at multiple predetermined pressure-specific bladder capacities may be more clinically meaningful
- Subjective patient/parental assessment (e.g., voiding diary) is critical to accurate evaluation of outcome
- Safety: 12 month data support generally well-tolerated technology
- Adverse events are as expected for this patient population
- Long-term follow up of these patients is ongoing

Reference cited: