Vertical Expandable Prosthetic Titanium Rib (VEPTR)

Policy Number: 7.01.110  
Origination: 9/2007  
Last Review: 10/2018  
Next Review: 10/2019

Policy
Blue Cross and Blue Shield of Kansas City (Blue KC) will provide coverage for a vertical expandable prosthetic titanium rib when it is determined to be medically necessary because the criteria shown below are met.

When Policy Topic is covered
Use of the vertical expandable prosthetic titanium rib is considered medically necessary in the treatment of progressive thoracic insufficiency syndrome due to rib and/or chest wall defects in infants/children between six months of age and skeletal maturity.

When Policy Topic is not covered
Use of the vertical expandable prosthetic titanium rib for all other conditions, including but not limited to the treatment of scoliosis in patients without thoracic insufficiency, is considered investigational.

Considerations
Skeletal maturity occurs at about age 14 for girls and age 16 for boys.

Due to complexity of thoracoplasty and the young age of the patient population undergoing such a procedure, implantation of the vertical expandable prosthetic titanium rib (VEPTR®) should be performed in specialized centers. Preoperative evaluation should require input from a pediatric orthopedist, a pulmonologist, and a thoracic surgeon. In addition, preoperative evaluation should require (when possible) a test for positive nutritional, cardiac, and pulmonary function.

Description of Procedure or Service

<table>
<thead>
<tr>
<th>Populations</th>
<th>Interventions</th>
<th>Comparators</th>
<th>Outcomes</th>
</tr>
</thead>
</table>
| Individuals:  
- With progressive thoracic insufficiency syndrome due to rib and/or chest wall defects in childhood | Interventions of interest are:  
- Vertical expandable prosthetic titanium rib | Comparators of interest are:  
- Respiratory supportive care | Relevant outcomes include:  
- Symptoms  
- Morbid events  
- Functional outcomes  
- Treatment-related mortality  
- Treatment-related morbidity |
The vertical expandable prosthetic titanium rib (VEPTR) is a curved rod placed vertically in the chest to help shape the thoracic cavity. It is being evaluated in skeletally immature patients with thoracic insufficiency syndrome (TIS) to support thorax and lung development and in pediatric patients with scoliosis without TIS to slow or correct curve progression.

For individuals who have progressive TIS who receive the VEPTR as a means of thoracoplasty, the evidence includes few case series. Relevant outcomes are symptoms, morbid events, functional outcomes, and treatment-related mortality and morbidity. TIS occurs in a limited patient population. For example, the Boston Center reported results on 31 children treated from 1999 to 2005. The natural history of progressive TIS is worsening pulmonary function and pulmonary insufficiency. Results from case series reported at different specialty centers have demonstrated improvement and/or stabilization in key measures with use of the VEPTR in progressive TIS. This improvement has been noted in measures related to thoracic structure (eg, Cobb angle for those with scoliosis), growth of the thoracic spine and lung volumes, and stable or improved ventilatory status. While pulmonary function testing is difficult to track in patients suffering with TIS, one study managed to demonstrate an age-specific increase in forced vital capacity; further still, that same study was able to report a final forced vital capacity in the range of 50% to 70% of predicted value. Given the usual disease course of worsening thoracic volume and ventilatory status, the stabilization and/or improvement in these measures would be highly unlikely if not for the intervention. Taken together, these outcome measures demonstrate the positive impact of utilizing the VEPTR technology. The evidence is sufficient to determine that the technology results in a meaningful improvement in the net health outcome.

For individuals with early-onset scoliosis without TIS who receive the VEPTR as a means of thoracoplasty, the evidence includes few case series. Relevant outcomes are symptoms, morbid events, functional outcomes, and treatment-related mortality and morbidity. The VEPTR is being evaluated for curves greater than 45° in infants and juveniles without thoracic insufficiency. Similar to TIS, very limited data are available on the use of the VEPTR for early-onset scoliosis without thoracic insufficiency; additionally, little is known about the disease progression of early-onset scoliosis, and therefore little is known regarding the risk-benefit tradeoff of the VEPTR® surgery. The evidence is insufficient to determine the effects of the technology on health outcomes.
Background
Thoracic insufficiency syndrome (TIS) is the inability of the thorax to support normal respiration or lung growth. (1) The condition results from serious defects affecting the ribs or chest wall, such as severe scoliosis, rib fusion (which may accompany scoliosis), and various hypoplastic thorax syndromes, such as Jeune syndrome and Jarcho-Levin syndrome. Spine, chest, and lung growth are interdependent (2). While the coexistence of chest wall and spinal deformity is well-documented, this effect on lung growth is not completely understood.

Progressive TIS includes respiratory insufficiency, loss of chest wall mobility, worsening 3-dimensional thoracic deformity, and/or worsening pulmonary function tests. As a child grows, progressive thoracic deformity and rotation toward the concave side occurs with worsening respiratory compromise. This progression is often accompanied by a need for supplemental oxygen and can require mechanical ventilation.

While spinal fusion is one approach to treatment, it may not be successful and also may limit growth (lengthening) of the spine.

The vertical expandable prosthetic titanium rib (VEPTR) is a curved rod placed vertically in the chest that helps to shape the thoracic cavity. It is positioned either between ribs or between the ribs and either the spine or pelvis. The device is designed to be expanded every 4 to 6 months as growth occurs and also to be replaced if necessary. Some patients require multiple devices.

Regulatory Status
A VEPTR initially received approval from FDA under a humanitarian device exemption for the treatment of TIS in skeletally immature patients.3 TIS is defined as the inability of the thorax to support normal respiration or lung growth. In 2014, FDA cleared the VEPTR through the 510(k) process. The VEPTR/VEPTR II device is indicated for skeletally immature patients with severe progressive spinal deformities and/or 3-dimensional deformity of the thorax associated with or at risk of TIS. This would include patients with progressive congenital, neuromuscular, idiopathic, or syndromic scoliosis.

For the purpose of identifying potential TIS patients, the categories in which TIS patients fall are as follows:

- Flail chest syndrome
- Rib fusion and scoliosis
- Hypoplastic thorax syndrome, including,
  - Jeune's syndrome
  - Achondroplasia
  - Jarcho-Levin syndrome
  - Ellis van Creveld syndrome
Rationale
This evidence was created in April 2007 and has been updated regularly with searches of the MEDLINE database. The most recent literature update was performed through February 5, 2018.

Evidence reviews assess the clinical evidence to determine whether the use of a technology improves the net health outcome. Broadly defined, health outcomes are length of life, quality of life, and ability to function—including benefits and harms. Every clinical condition has specific outcomes that are important to patients and to managing the course of that condition. Validated outcome measures are necessary to ascertain whether a condition improves or worsens; and whether the magnitude of that change is clinically significant. The net health outcome is a balance of benefits and harms.

To assess whether the evidence is sufficient to draw conclusions about the net health outcome of a technology, 2 domains are examined: the relevance and the quality and credibility. To be relevant, studies must represent one or more intended clinical use of the technology in the intended population and compare an effective and appropriate alternative at a comparable intensity. For some conditions, the alternative will be supportive care or surveillance. The quality and credibility of the evidence depend on study design and conduct, minimizing bias and confounding that can generate incorrect findings. The randomized controlled trial is preferred to assess efficacy; however, in some circumstances, nonrandomized studies may be adequate. Randomized controlled trials are rarely large enough or long enough to capture less common adverse events and long-term effects. Other types of studies can be used for these purposes and to assess generalizability to broader clinical populations and settings of clinical practice.

Thoracic insufficiency occurs in a limited patient population, and the literature on the use of the vertical expandable prosthetic titanium rib (VEPTR) consists mostly of case series from single institutions (some series are from specialized pediatric centers); no comparative trials have been identified.

Thoracic Insufficiency Syndrome
Data submitted to the U.S. Food and Drug Administration (FDA) on thoracic insufficiency syndrome (TIS) include an initial feasibility study involving 33 patients and a subsequent prospective study of 224 patients (214 with baseline data) at 7 study sites. Of these, 94 had rib fusion, 93 had hypoplastic thoracic syndrome, 46 had progressive scoliosis, and 14 had flail chest as a cause of their TIS. Three- and 5-year follow-up rates for the multicenter study were approximately 95%. Of the 247 patients enrolled in either study, 12 (4.8%) patients died, and 2 withdrew. None of the deaths, as determined by investigators, were related to the VEPTR. Because standard pulmonary function testing was not possible for most of this population, an assisted ventilatory rating was used to assess impact on respiratory status. The assisted ventilatory rating ranged from 0 (unassisted breathing on room air) to 4 (full-time ventilatory support). In the multicenter prospective study, the assisted ventilatory rating outcome improved or
stabilized for 93% of the patients. Data were not reported for the number of patients who were no longer dependent on a ventilator.

Campbell (2004), who developed the VEPTR, and colleagues reported on 27 patients who had surgery for TIS and at least 2 years of follow-up data; this series was based on 41 patients treated between 1990 and the study reporting. Entry criteria for this study were acceptance by pediatric general surgeon, pediatric pulmonologist, and a pediatric orthopedist; age 6 months to skeletal maturity; progressive TIS; more than 10% reduction in height of the concave hemithorax; and 3 or more anomalous vertebrae, with 3 or more fused ribs at the apex of the deformity. Patients were followed for an average of 3.2 years (range, 2-12 years). Before surgery, the mean annual rate of progression was 15° per year (range, 2°-50° per year). Following surgery, the Cobb angle (of scoliosis) improved from 74° to a final value of 49°. Spine growth was at a rate of 0.8 cm per year. (Normal spinal growth is 0.6 cm/year for ages 5-10 years.) The final forced vital capacity (FVC) was 49% of predicted value in the 19 children who could complete pulmonary function tests. Preoperatively, 1 patient required continuous positive airway pressure, and another needed supplemental oxygen for ventilatory support at final follow-up. Another publication (2013) from this group reported average 40.7-month follow-up (range, 25-78 months) in 24 children with nonsyndromic congenital scoliosis. Twenty-three (95.8%) children had associated rib fusions, and the average age at surgery was 3.3 years (range, 0.7-12.5 years). With a mean of 5 expansion surgeries per patient (range, 1-10), the mean Cobb angle improved by 8.9° and mean thoracic height improved by 3.41 cm. Eight (33%) patients had a total of 16 adverse events, all of which required surgery.

In another series, Gadepalli et al (2011) examined growth and pulmonary function in 26 children who received a VEPTR between 2006 and 2010. The children underwent 29 insertions and 57 expansions, with an average of 3 surgeries per child. Each procedure required an average 0.97 days in the intensive care unit and 4.41 days in the hospital. The mean Cobb angle improved by 29%, from 64.7° preoperatively to 46.1° postoperatively. Lung volumes measured by yearly thoracic computed tomography scans were similar when corrected for age. Pulmonary function tests were performed every 6 months in patients (n=12) who were not ventilator-dependent and could cooperate with the procedure. Pulmonary function tests showed no significant change from baseline to follow-up in percent predicted values for forced expiratory volume in 1 second (54.6 L vs 51.8 L), FVC (58.1 L vs 55.9 L), or residual volume (145.3 L vs 105.6 L), all respectively. Reoperation was required for 14 complications, 4 for chest tube placement (pneumothorax), 1 for seroma drainage, 6 for hardware removal (for infection), and 3 for hardware repositioning (for dislodgement). Another 22 complications were treated nonoperatively.

Emans et al (2005) reported results for patients with TIS who underwent the procedure at a single children’s hospital from 1999 to 2005. Thirty-one patients with fused ribs and TIS were treated; 4 patients had prior spinal arthrodesis with continued progression of deformity. Before surgery, all patients showed progressive spinal deformity, progressive chest deformity, or progressive
hemithoracic constriction. The mean age was 4.2 years, and mean follow-up was 2.6 years (range, 0.5-5.4 years). A 3-member team selected patients for surgery, and cardiac function was evaluated preoperatively. Lengthening of the VEPTR was planned for every 4 to 6 months but often was longer due to intercurrent illness or difficulty with travel. The mean number of device lengthenings was 3.5 (range, 0-10). Six patients had device exchanges for growth. In 30 patients, spinal deformity was controlled, and growth continued (1.2 cm/y) in the thoracic spine during treatment at rates similar to normal children. In this study, final FVC was 73.5% of predicted levels. Prior to the procedure, 2 patients were on ventilators and 3 patients required oxygen; at final follow-up, 1 patient required oxygen. Lung volume (measured by computed tomography scan) in the operated lung increased from 157 cm$^3$ preoperatively to 326 cm$^3$ at the final follow-up visit.

Motoyama et al (2006) from a children’s hospital reported on 10 patients with TIS. Using a special portable pulmonary function test device, they reported on lung function in 10 children who had a VEPTR. Median age was 4.3 years (range, 1.8-9.8 years) at first test, and patients were followed an average of 22 months (range, 7-33 months). At baseline, FVC showed a moderate-to-severe decrease (69% of predicted), indicating the presence of significant restrictive lung defect. FVC increased significantly over time, with an average rate of 26.8% per year, similar to that of healthy children of comparative ages. In terms of percent predicted values, FVC did not change significantly between the baseline and last test (70.3%), indicating that, in most children studied, lung growth kept pace with body growth.

A series of 22 patients from another children’s hospital was published in 2007. Seven (19%) of the 36 the VEPTR units placed required revision and 10 of 22 children reported better activity levels while 2 of 22 children reported better respiratory function.

Other series have discussed weight gain after use of VEPTR in TIS or early changes in pulmonary function.

**Section Summary: Thoracic Insufficiency Syndrome**
The evidence evaluating use of VEPTR thoracoplasty to treat children with progressive TIS due to rib and/or chest wall defects consists of a few case series. TIS occurs in a limited patient population. For example, the Boston Center reported results on 31 children treated from 1999 to 2005. The natural history of progressive TIS is worsening pulmonary function and pulmonary insufficiency. Results from the case series reported by different specialty centers have demonstrated improvement and/or stabilization in key measures with use of the VEPTR in progressive TIS. This improvement has been noted in measures related to thoracic structure (eg, Cobb angle for those with scoliosis), growth of the thoracic spine and lung volumes, and stable or improved ventilatory status. While pulmonary function testing is difficult to track in patients suffering with TIS, a study has demonstrated an age-specific increase in FVC; further still, that same study reported a final FVC in the range of 50% to 70% of predicted value. Given the usual disease course of worsening thoracic volume and ventilatory status, the
stabilization and/or improvement in the clinical measures outlined above would be highly unlikely if not for the intervention. Taken together, these outcomes demonstrate the positive impact of using the VEPTR technology.

**Scoliosis Without TIS**

White et al (2011) reported on the off-label use of spine-to-spine VEPTR to treat spinal deformity in 14 children without chest wall abnormalities. The indications for the dual spine-to-spine rods were absence of a primary chest wall deformity, progression of spinal deformity to a Cobb angle of greater than 50°, and migration of a previously placed proximal rib anchor or a prior non-VEPTR growing rod to the point of loss of stable fixation. At final follow-up (24-48 months), there was an improvement in the Cobb angle from 74° to 57°, an increase in T1-S1 height from 260 to 296 mm, and no significant change in kyphosis. Complications occurred in 6 (43%) of 14 patients and included 3 rod fractures in 2 patients, 3 superficial infections, and 1 case of prominent hardware that threatened skin integrity. As noted by authors, while results were similar to those obtained with other growing rods, “the high complication rates, need for multiple procedures in growing children, and small relative gains in radiographic parameters still challenge proof of the efficacy of all such treatment methods.”

Using data from a prospective registry, Farley et al (2014) compared treatment of congenital scoliosis using VEPTR (n=22) with treatment using spinal fusion (n=27) and observation (n=184). Function, pain, and mental health status were measured with the 22-item Scoliosis Research Society questionnaire. Compared with the observation group, the VEPTR group had higher total and image scores at the second and third visits and higher function scores at the third and fourth visits. Interpretation of this study is limited due to confounding factors, including age at treatment, unknown comorbidities, and the rationale for treatment selection.

**Section Summary: Scoliosis Without TIS**

The evidence evaluating use of VEPTR thoracoplasty to treat young children with early-onset scoliosis without TIS consists of a few case series. The VEPTR is being evaluated for curves greater than 45° in infants and juveniles without thoracic insufficiency. Similar to TIS, very limited data are available on the use of the VEPTR for early-onset scoliosis without thoracic insufficiency; additionally, little is known about the disease progression of early-onset scoliosis, and therefore little is known regarding the risk-benefit tradeoff of the VEPTR surgery.

**Adverse Events**

Complications that occur with VEPTR need to be considered by practitioners and families when discussing this procedure. Information on complications has been summarized using data from the Food and Drug Administration review and the articles by Campbell and Emans. Up to 25% of patients may experience device migration, including rib erosion. Approximately 10% of patients had infection-related complications. Brachial plexus injury or thoracic outlet syndrome occurred in 1% to 7% of these series. Skin sloughing was reported in 4 (15%) patients in the study by Campbell. In a single-center series, Waldhausen et al (2016)
reported on device-related complications in 22 of 65 patients treated for TIS over a 13-year period.\textsuperscript{14}

**Summary of Evidence**

For individuals who have progressive TIS due to rib and/or chest wall defects in childhood who receive VEPTR thoracoplasty, the evidence includes a few case series. Relevant outcomes are symptoms, morbid events, functional outcomes, and treatment-related mortality and morbidity. TIS occurs in a limited patient population. For example, the Boston Center reported results on 31 children treated from 1999 to 2005. The natural history of progressive TIS is worsening pulmonary function and pulmonary insufficiency. Results from case series reported at different specialty centers have demonstrated improvement and/or stabilization in key measures with use of the VEPTR in progressive TIS. This improvement has been noted in measures related to thoracic structure (eg, Cobb angle for those with scoliosis), growth of the thoracic spine and lung volumes, and stable or improved ventilatory status. While pulmonary function testing is difficult to track in patients suffering with TIS, a study has demonstrated an age-specific increase in forced vital capacity; further still, that same study reported a final forced vital capacity in the range of 50\% to 70\% of predicted value. Given the usual disease course of worsening thoracic volume and ventilatory status, the stabilization and/or improvement in the clinical measures outlined above would be highly unlikely if not for the intervention. Taken together, these outcomes demonstrate the positive impact of using the VEPTR technology. The evidence is sufficient to determine that the technology results in a meaningful improvement in the net health outcome.

For individuals with early-onset scoliosis without TIS who receive VEPTR thoracoplasty, the evidence includes a few case series. Relevant outcomes are symptoms, morbid events, functional outcomes, and treatment-related mortality and morbidity. The VEPTR is being evaluated for curves greater than 45\° in infants and juveniles without thoracic insufficiency. Similar to TIS, very limited data are available on the use of the VEPTR for early-onset scoliosis without thoracic insufficiency; additionally, little is known about the disease progression of early-onset scoliosis, and therefore little is known regarding the risk-benefit tradeoff of the VEPTR surgery. The evidence is insufficient to determine the effects of the technology on health outcomes.

**Supplemental Information**

**Practice Guidelines and Position Statements**

No guidelines or statements were identified.

**U.S. Preventive Services Task Force Recommendations**

Not applicable.
Medicare National Coverage
There is no national coverage determination. In the absence of a national coverage determination, coverage decisions are left to the discretion of local Medicare carriers.

Ongoing and Unpublished Clinical Trials
Some currently unpublished trials that might influence this review are listed in Table 1.

Table 1. Summary of Key Trials

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<th>NCT No.</th>
<th>Trial Name</th>
<th>Planned Enrollment</th>
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<td>Unpublished</td>
<td>VEPTR Implantation to Treat Children With Early Onset Scoliosis Without Rib Abnormalities: A Prospective Multicenter Study</td>
<td>250</td>
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</table>

NCT: national clinical trial.

a Denotes industry-sponsored or cosponsored trial.

References

**Billing Coding/Physician Documentation Information**
There is no specific code for this procedure. The procedure would most likely be reported with the unlisted code 22899.

<table>
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<th>ICD-10 Codes</th>
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<tbody>
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<td><strong>M41.00-</strong></td>
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<td><strong>M41.9</strong></td>
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<td>Q76.3</td>
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<td>Q77.2</td>
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<td>Q87.2</td>
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**ICD-10 Codes**

- **M41.00-M41.9**: Scoliosis code range
- **Q76.3**: Congenital scoliosis due to congenital bony malformation
- **Q76.6**: Other congenital malformations of ribs (includes congenital absence of rib and congenital fusion of ribs)
- **Q77.2**: Osteochondrodysplasia with defects of growth of tubular bones and spine; short rib syndrome (includes Asphyxiating thoracic dysplasia [Jeune])
- **Q87.2**: Congenital malformation syndromes predominantly involving limbs (includes VATER syndrome)

**Additional Policy Key Words**

N/A

**Policy Implementation/Update Information**

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