DISCLAIMER/INSTRUCTIONS FOR USE

Medical Policy provides general guidance for applying Blue Cross of Idaho benefit plans (for purposes of Medical Policy, the terms “benefit plan” and “member contract” are used interchangeably). Coverage decisions must reference the member specific benefit plan document. The terms of the member specific benefit plan document may be different than the standard benefit plan upon which this Medical Policy is based. If there is a conflict between a member specific benefit plan and the Blue Cross of Idaho’s standard benefit plan, the member specific benefit plan supersedes this Medical Policy. Any person applying this Medical Policy must identify member eligibility, the member specific benefit plan, and any related policies or guidelines prior to applying this Medical Policy. Blue Cross of Idaho Medical Policies are designed for informational purposes only and are not an authorization, explanation of benefits or a contract. Receipt of benefits is subject to satisfaction of all terms and conditions of the member specific benefit plan coverage. Blue Cross of Idaho reserves the sole discretionary right to modify all its Policies and Guidelines at any time. This Medical Policy does not constitute medical advice.

POLICY

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 and children between 6 months of age and skeletal maturity.

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.

POLICY GUIDELINES

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.

CODING

There is no specific CPT code for this procedure. The procedure would most likely be reported with the unlisted code 22899.

BENEFIT APPLICATION

BLUECARD/NATIONAL ACCOUNT ISSUES

State or federal mandates (e.g., Federal Employee Program) may dictate that certain U.S. Food and Drug Administration–approved devices, drugs, or biologics may not be considered investigational, and thus
these devices may be assessed only by their medical necessity.

**BACKGROUND**

**Thoracic Insufficiency Syndrome**

Thoracic insufficiency syndrome (TIS) is the inability of the thorax to support normal respiration or lung growth. The condition results from serious defects affecting the ribs or chest wall (eg, severe scoliosis with rib absence or rib fusion) and various hypoplastic thorax syndromes (eg, Jeune syndrome, Jarcho-Levin syndrome). Spine, chest, and lung growth are interdependent. 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.

**Treatment**

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

The vertical expandable prosthetic titanium rib (VEPTR) device is a curved rod placed vertically in the chest that helps to stabilize and shape the thoracic cavity. It is positioned either between ribs or between the ribs and either the spine or pelvis. The VEPTR may be described as “rib-based” growth-sparing instrumentation, which is compared with “spine-based” growing rods for Cobb angle correction. The VEPTR device is designed to be expanded every 4 to 6 months as growth occurs and to be replaced if necessary. Some patients require multiple devices.

**Regulatory Status**

The VEPTR® (DePuy Synthes Spine, Raynham, MA) was initially cleared for marketing by the U.S. Food and Drug Administration through a humanitarian device exemption for the treatment of TIS in skeletally immature patients. In 2014, the VEPTR® was cleared for marketing by the Food and Drug Administration through the 510(k) process. The VEPTR® and VEPTR II™ devices are 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.

To identify potential TIS patients, the following categories are used:

- Flail chest syndrome
- Rib fusion and scoliosis
- Hypoplastic thorax syndrome, including
  - Jeune syndrome
  - Achondroplasia
  - Jarcho-Levin syndrome
  - Ellis-van Creveld syndrome.

Food and Drug Administration product code: MDI.
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, 2019.

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.

Clinical Context and Therapy Purpose

The purpose of the vertical expandable prosthetic titanium rib in patients who have progressive thoracic insufficiency syndrome or early-onset scoliosis without thoracic insufficiency syndrome is to provide a treatment option that is an alternative to or an improvement on existing therapies.

The following PICOTS were used to select literature to inform this review.

Patients

The relevant populations of interest are children who have progressive thoracic insufficiency syndrome and young children with early-onset scoliosis without thoracic insufficiency syndrome.

Interventions

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

Comparators

For progressive thoracic insufficiency syndrome, relevant comparators include respiratory supportive care.

For early onset scoliosis, relevant comparators include spinal fusion and bracing.

Outcomes
The general outcomes of interest are symptoms, morbidity, treatment-related mortality, and treatment-related morbidity.

Thoracic Insufficiency Syndrome

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.

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 cm³ preoperatively to 326 cm³ 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

Original Policy Date: April 2007
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.

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 (e.g., 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>Trial Name</th>
<th>Planned Enrollment</th>
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<td>Vertical Expandable Prosthetic Titanium Rib (VEPTR) for Thoracic Insufficiency Syndrome</td>
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<td>VEPTR Implantation to Treat Children With Early Onset Scoliosis Without Rib Abnormalities: A Prospective Multicenter Study</td>
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NCT: national clinical trial.
Denotes industry-sponsored or cosponsored trial.

ESSENTIAL HEALTH BENEFITS

The Affordable Care Act (ACA) requires fully insured non-grandfathered individual and small group benefit plans to provide coverage for ten categories of Essential Health Benefits (“EHBs”), whether the benefit plans are offered through an Exchange or not. States can define EHBs for their respective state.

States vary on how they define the term small group. In Idaho, a small group employer is defined as an employer with at least two but no more than fifty eligible employees on the first day of the plan or contract year, the majority of whom are employed in Idaho. Large group employers, whether they are self-funded or fully insured, are not required to offer EHBs, but may voluntary offer them.

The Affordable Care Act requires any benefit plan offering EHBs to remove all dollar limits for EHBs.

REFERENCES


CODES

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<td>Congenital scoliosis due to congenital bony malformation</td>
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<td>Other congenital malformations of ribs (includes congenital absence of rib and congenital fusion of ribs)</td>
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<td>Congenital malformation syndromes predominantly involving limbs (includes VATER syndrome)</td>
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<td>Surgery, upper bones, replacement, rib (right or left), synthetic substitute; open, percutaneous and percutaneous endoscopic approach codes</td>
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Type of Service: Surgery
Place of Service: Outpatient/Inpatient

POLICY HISTORY

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Original Policy Date: April 2007
February 5, 2019; no references added. Policy statements unchanged.