



**Deflazacort, Eteplirsen, and Golodirsen for Duchenne Muscular  
Dystrophy:  
Effectiveness and Value  
Modeling Analysis Plan**

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# 1. Approach

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The purpose of this economic evaluation is to assess the cost-effectiveness of deflazacort (Emflaza®), eteplirsen (Exondys 51™), and golodirsen for treating Duchenne Muscular Dystrophy (DMD). We anticipate that modified societal costs associated with DMD will be substantial relative to the condition’s health care sector costs, and hence we expect to present base-case results from both health care sector and modified societal perspectives, aligning with ICER’s [Value Assessment Framework for Ultra Rare Diseases](#). A decision-analytic model will be developed to project lifetime costs, life years (LYs) and quality-adjusted life years (QALYs), discounted at 3%, for patients with DMD. Where there is insufficient evidence of a treatment effect, the model will be used to project threshold treatment effects for the treatments to be considered cost-effective at value thresholds of \$50,000, \$100,000 and \$150,000 per QALY gained. The models will be developed using Microsoft Excel 2016 (Redmond, WA).

Please also refer to the details on the systematic review of the clinical evidence on this [topic](#).

## 2. Methods

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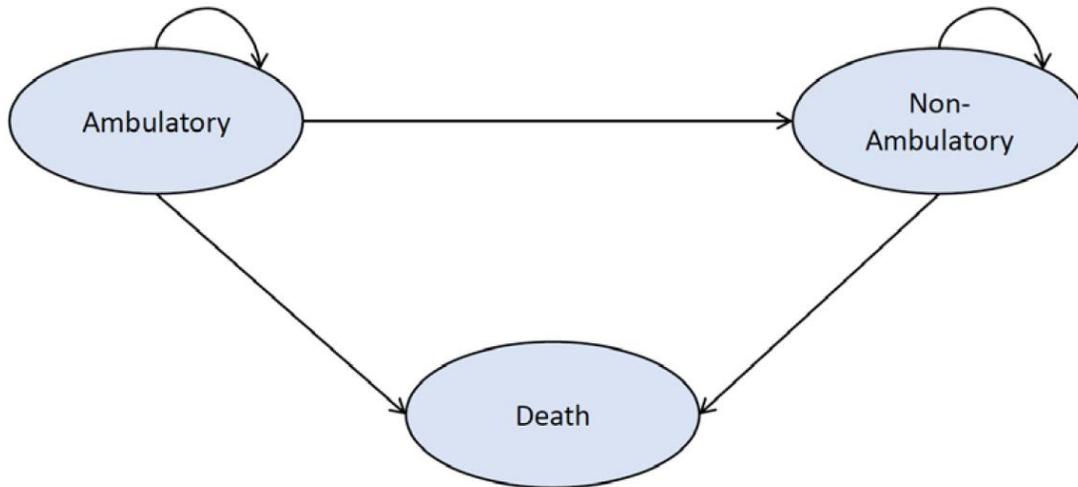
### 2.1 Overview and Model Structure

We will develop a partitioned survival model for this evaluation, informed by key clinical trials, cohort studies, and prior relevant studies related to economic modeling in DMD.<sup>1-6</sup> Figure 1 below illustrates the basic structure of the model.

The model will focus on an intention-to-treat analysis using a hypothetical cohort of patients with DMD. The model will use annual time periods, based on what was observed in prior published economic models as well as the clinical and longitudinal data, and will be run over a lifetime time horizon. Costs and outcomes will be discounted at 3% per year. We will evaluate deflazacort versus prednisone in the treatment of DMD. We will also evaluate eteplirsen, and golodirsen as add-on therapies to corticosteroids in the treatment of DMD for the subset of patients eligible to receive these new drugs, pending data availability. If insufficient data are available for assessing treatment effects, we will use the model to estimate threshold treatment effects that would be necessary for the treatment to achieve specific value thresholds such as \$150,000 per QALY.

For each treatment regimen, patients will enter the model in the “ambulatory” health state at age five years. From the “ambulatory” health state, patients will transition to “non-ambulatory” or “death” health states based on projected survival curves for these health states estimated from historical data and clinical trial evidence for patients with DMD.<sup>2</sup>

**Figure 1. Model Schematic**



## 2.2 Key Model Choices and Assumptions

Medical and non-medical costs, patient utility, and caregiver utility will depend on the patient’s health state and will be calculated for the entire modeled cohort on an annual basis. Treatment effects in the model can impact costs and QALYs by extending time in the ambulatory health state and delaying time until death and/or by having different adverse event (AE) profiles.

Our model will include several assumptions stated below in Table 1.

**Table 1. Key Model Assumptions**

Assumption	Rationale
<b>Patients on prednisone will transition between “ambulatory”, “non-ambulatory” and “death” health states following the survival curves projected in a prior analysis which itself was based on international clinical trial data and historical data for patients diagnosed with DMD and receiving steroids.<sup>2</sup></b>	The best available estimate for current health trajectories associated with patients on prednisone is the Hercules analysis projecting the health states of DMD patients on steroids. Those projections match generally with age at loss of ambulation found in <a href="#">MD Starnet</a> for US patients.
<b>Costs and utilities will be for ambulatory and non-ambulatory health states based on prior survey results that had been categorized into early and late ambulatory, and early and late non-ambulatory. Hence, the relative proportion of ambulatory patients in early and late ambulation and in early and late non-ambulation after losing ambulation</b>	Available survey data on costs and utilities had been divided into early and late ambulatory, and early and late non-ambulatory based solely on age. There are insufficient data to differentiate treatment effects on what this past survey had defined as early or late ambulation or early or late non-ambulation. We will use constant costs and utilities for time in ambulatory and non-ambulatory, combining the early and late values based on the relative proportion of patients in the

Assumption	Rationale
<b>is assumed to be the same in all treatments.</b>	early and late stages that contributed to the original estimates. <sup>7</sup>
<b>Treatment effects in time to non-ambulation will be modeled using direct rightward shifts (i.e., parallel shifts) in the non-ambulation survival curve being used in the model with and without equivalent shifts in the mortality curve based on years gained during ambulation found from the literature.</b>	In the absence of long-term data, rightward shifts in the ambulation and mortality survival curve are a reasonable approximation of likely long-term effects related to shorter term evidence in increased time in ambulation. Similar assumptions have been made in past models of DMD. <sup>3</sup>
<b>Treatment effects based on the six-minute walk test will result in a parallel shift upwards, at that age, of the patterns seen in historical data and fitted curves from a past comprehensive study on historical six minute walk test data by age will be used to project changes in number of years ambulatory defined as a six minute walk test above zero.<sup>8</sup></b>	In extrapolating effects on time to being non-ambulatory, there are not enough data to establish the precise impact on the trajectory of distances observed in the six-minute walk test from an increase at a particular age. We feel a direct upwards shift equal to the observed treatment effect at the age where the treatment effect was found and then thereafter following historical patterns of decline, would be a reasonable assumption.
<b>The relative proportion of modified societal perspective and health care sector perspective costs are the same in the ambulatory and non-ambulatory health states.</b>	Cost estimates by specific category that allow separation of costs into a health sector and a modified societal perspective are not available across health states, only in terms of the overall average costs. Changes in total direct costs are available across health states by country and the ratio of non-ambulatory to ambulatory in those costs for the US are used to adjust the costs across health states for available health system and societal perspective costs. <sup>7</sup>
<b>Patients are diagnosed and begin treatment at five years of age.</b>	Available evidence suggests diagnosis occurs around five years of age and patients do best with long-term treatment with steroids beginning at age five.
<b>Cataracts result in an office visit and a disutility of 0.05 for the proportion of patients experiencing a cataract each year. In addition, a small proportion of cataracts each year will be assigned a cost of surgery.</b>	We could not identify disutility estimates for cataracts in the literature. There was an estimated disutility of cataract surgery of 0.08 in a UK study. <sup>9</sup> However, as a conservative approach we will use a 0.05 disutility as an upper bound on potential QALY effects. There are no estimates of rates of cataract surgery among DMD patients. Costs related to surgery will be projected to the DMD population with cataracts based on data of the general prevalence of cataract surgery in the US. <sup>10</sup>
<b>If sufficient evidence is found, weight gain will result in a utility decrement of 0.05.</b>	Measures of weight gain in the literature are inconsistently defined. In addition, the few available estimates of disutility for weight gain for children in the literature, were smaller (less than 0.01 based on the PEDSQL). <sup>11,12</sup> However, as a conservative approach, we believe 0.05 would be a reasonable disutility as an upper bound on potential QALY effects from lowering this AE.

## 2.3 Target Populations

The population of focus for the economic evaluation will be patients in the United States (US) diagnosed with DMD. For model projections related to eteplirsen and golodirsen, the population will be those eligible to receive those treatments. In the model, patients will begin at the age of five years and outcomes will be projected over a lifetime.

## 2.4 Interventions

The list of interventions was developed with input from patient organizations, clinicians, manufacturers, and payers. Choices of comparators were chosen to best reflect real world treatment decisions likely to be made by clinicians. The list of interventions and comparator is presented below:

- Deflazacort (Emflaza®)
- Eteplirsen (Exondys 51™)
- Golodirsen
- Prednisone

One comparison will be between deflazacort and prednisone. Eteplirsen and golodirsen will also be considered as additional treatment to corticosteroids.

## 2.5 Input Parameters

### Clinical Inputs

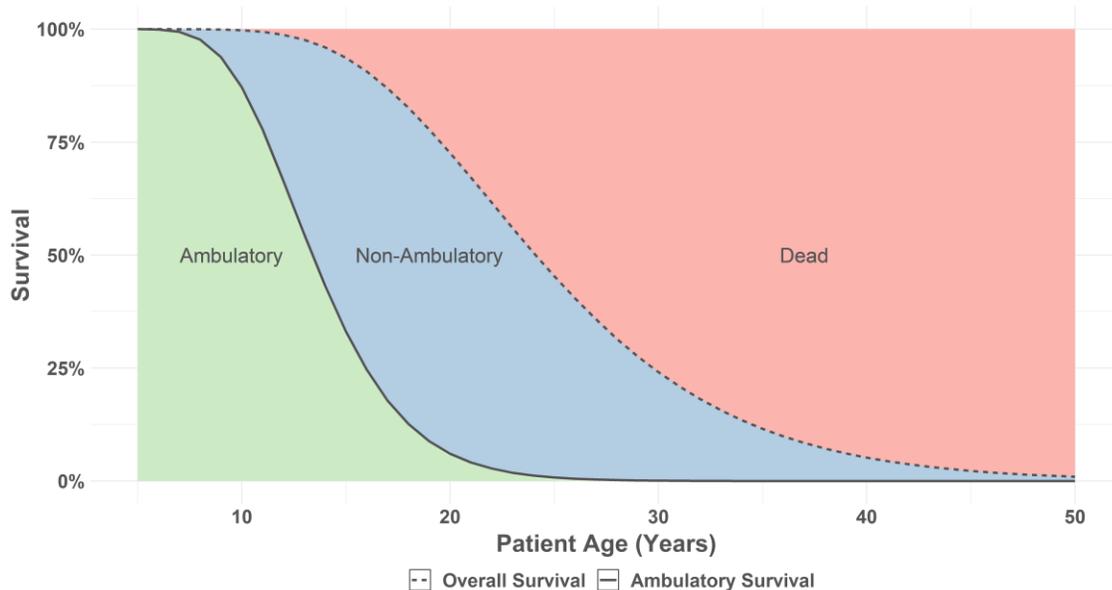
Clinical inputs will come from selected studies. Due to the inadequate data, a network meta-analysis (NMA) from available trials measuring treatment efficacy is not possible between any of the stated interventions. We are exploring the possibility of conducting a meta-analysis of trials and observational data to estimate the relative efficacy and safety of deflazacort and prednisone.

### *Transition Probabilities*

Survival curves on time to non-ambulation and time to death for patients on steroids will be used from a recent research project.<sup>2</sup> In that study, Kaplan Meir (KM) curves for loss of ambulation as well as between non-ambulation and death were projected for steroid users based on the following: 1) available survival data for DMD patients with and without steroid use, and 2) past work regarding modeling DMD.<sup>1,2</sup> These curves were adjusted for censoring and fit with functional forms; a treatment effect for steroids was estimated and the curves were projected.

Kaplan Meier curves will be digitized and parametric curve functions will be fit using an approach described in a previous published study on estimating survival curves.<sup>13</sup> The model curves will include the distributional forms Weibull, exponential, log-normal, log-logistic, gamma, and Gompertz. The base-case parametric functions for each comparator will be selected based on best model fit using Akaike information criterion (AIC) values and visual comparison. Fitted curves will be used to directly partition patients into ambulatory, non-ambulatory, and death states across time (see Figure 2 below). Note that prior studies related to cost and utility surveys, and models of DMD, distinguished early and late ambulatory and early and late non-ambulatory based purely on age-related definitions.<sup>1,2</sup> Our model will combine the early and late states of ambulation and non-ambulation as there is currently no evidence for how treatments impact the time spent in early or late ambulation or in early or late non-ambulation analogously to what was used in the past survey and analytical studies we borrow from. In combining those states, we will use the relative proportion of US patients in early and late ambulatory and early and late non-ambulatory states as seen in the original survey study we use for health state specific costs and utilities,<sup>7</sup> described further below. An assumption here is that treatments may extend time to loss of ambulation, but they do not change the proportion of time spent in “early” versus “late” ambulation and similarly affect the non-ambulatory state.

**Figure 2. Comparator (Prednisone) Survival Curves\* of Probability of Being in Each State at Each Age Beginning at Age 5**



\*Survival curves are digitized from a prior analysis and combines early and late non-ambulatory states.<sup>2</sup>

### ***Discontinuation***

There is evidence that a substantial proportion (39%) of patients discontinue steroid treatment upon reaching a non-ambulatory health state.<sup>14</sup> Available discontinuation rates will be incorporated in the model for estimating treatment-related costs.

### ***Mortality***

Age-dependent mortality for patients with DMD is net estimated in the survival curve described above in Figure 2. These match generally with age at loss of ambulation data and other demographic data in in the US and the modeled mortality in a prior DMD analysis.<sup>1</sup>

### ***Adverse Events***

In our preliminary search within the published literature, we found several cohort studies pertaining to AEs related to deflazacort and prednisone. As stated earlier, pending data availability and fit, AE inputs in the model may be informed by a potential NMA, meta-analysis, or selected results from the cohort studies.

There were no reported significant serious adverse events (SAEs) for eteplirsen or golodirsen. The comparison of deflazacort and prednisone will include the modeled impact of differences in rates of cataracts seen when using these drugs. Given a paucity of estimates in the literature and none specifically recorded in children, we used an assumed disutility of 0.05 per year for having a cataract.

For the cost of cataract surgery, we will use cost estimates from the literature<sup>15</sup> and inflate them to 2018 dollars as per the [ICER Reference Case](#). We will also use the adverse event rates of developing cataracts when using prednisone and deflazacort (see Table 2).<sup>10</sup> We will calculate the cost of cataract surgery for the cohort by applying the proportion of those diagnosed with cataract who underwent cataract surgery. If we are able to find enough evidence to include it, we will define unwanted weight gain as greater than or equal to 10% of current weight. We have not identified any literature to suggest a health care cost associated with unwanted weight gain for the pediatric patient. Thus, we would assume no cost associated with weight gain. However, we would explore a range of costs in the sensitivity analyses. For the weight gain adverse events, we will use available rates from an RCT of deflazacort and prednisone or a combination of this and other trials.<sup>16</sup> If evidence of other relevant AEs are found we will incorporate estimates of their impact into the model.

**Table 2. Adverse Events**

Parameter	Rate	Disutility	Cost	Source
Cataracts	Prednisone: 0.17% per year Deflazacort: 0.36% per year	0.05	\$856	Brown, 2013 Rice, 2018

**Health State Utilities**

Health state utilities will be based on a prior study that included survey data on US DMD patients (see Table 3).<sup>7</sup> We will combine the early and late ambulatory states into the ambulatory state by weighting the early and late states based on the proportions of patients seen in those states, specifically 30.38% in early and 69.62% in late, in the US study population.<sup>7</sup> The same will be done for the non-ambulatory states using 38.89% for early and 61.11% for late. The health care sector perspective base case will use only patient utilities and related QALYs. The modified societal perspective will consider patient and caregiver utilities available in survey data incorporating the Health Utility Index (HUI) using an assumption of one caregiver per patient.<sup>7</sup>

**Table 3. Ambulatory and Non-Ambulatory Health State Utility Scores**

Health State	Patient Utility	Caregiver Utility	Source
Ambulatory	0.61	0.845	Landfeldt 2014
Non-Ambulatory	0.38	0.800	Landfeldt 2014

**Drug Utilization**

Drug utilization will be based on recommended treatment guidelines as outlined in Table 4.

**Table 4. Drug Doses**

Generic Name (Brand Name)	Dose	Approval status	Source
Prednisone	Approximately 0.75 mg/kg/day administered orally	Generic, Used Off Label	Griggs, 2016
Deflazacort (EMFLAZA®)	Approximately 0.9 mg/kg/day administered orally	Approved	FDA Label
Eteplirsen (EXONDYS 51™)	100 mg/2 mL (50 mg/mL) in single-dose vial 500 mg/10 mL (50 mg/mL) in single-dose vial	Accelerated Approval Contingent on Verification of Clinical Benefit	FDA Label
Golodirsen (SRP-4053)	Dose Titration	Not Approved, Regulatory Action Date: August 19 <sup>th</sup> , 2019	Phase 1 / 2 Sarepta trial

## Cost Inputs

### Drug Costs

We will use the wholesale acquisition cost for prednisone and the Federal Supply Schedule (FSS) price for deflazacort. Since numerous generic forms of prednisone are available in the US market, we have used its average generic price across different dosing strengths of its oral tablet formulation. For deflazacort, we have currently calculated its price based on its tablet and suspension forms. Since eteplirsen is administered in a hospital or physician's office setting, we expect it to have a price mark-up. Adhering to the ICER Reference Case, we have included a price mark-up for eteplirsen, defined as the average wholesale price (AWP) minus 15% and adjusted to reflect hospital administration. In the absence of a price estimate for golodirsen, we will assume its price to be the same as that of eteplirsen. Note that all drugs are dosed by weight and hence will vary in the model across patient age based on expected weight in the model. Expected weight across patient age will itself be modeled from past information regarding weight of US DMD patients by age and/or [general estimates of weight by age in the US as available](#).<sup>17</sup> Please see Table 5 for more details and where we include annual treatment cost estimates for a 30-kg patient.

**Table 5. Drug Costs**

Intervention (Dosage)	WAC/Net	Net Annual Cost	Source
Prednisone (0.75mg/KG/Day)	\$0.05/mg	\$419*	Red Book
Deflazacort (0.9mg/KG/Day)	\$6.19/mg	\$61,004*	FSS Database
Eteplirsen 100 mg/2mL (50mg/mL) 500 mg/10mL (50mg/mL) (Dose 900mg/KG per week)	\$816/50mg/ML	\$763,776*	Red Book AWP -15% adjusted to reflect hospital administration
Golodirsen Pending	N/A	\$763,776*	Assumption (Same as Eteplirsen)

\*These estimates are for a 30-kg patient. Actual costs in the model will vary across age based on expected weight by patient age.

### Non-Drug Health Care Costs

The health state-specific costs will be based on a previous cross-sectional cost study that included US specific estimates.<sup>7</sup> All costs were inflated from 2012 to 2018 dollars per [ICER's Reference Case](#). We digitized specific mean per-patient cost of illness estimates from a detailed cost study in DMD<sup>7</sup> for each of the four health states: early ambulatory, late ambulatory, early non-ambulatory, and late non-ambulatory. To get healthcare perspective costs from the available data, we will assume a

constant proportion of modified societal costs made up by health system perspective costs across health states.

In addition, the model will combine the early and late ambulatory and the early and late non-ambulatory costs into ambulatory and non-ambulatory costs based on the proportion of US patients responding to the underlying survey (Table 6; see Health State Utilities section above where this same process was used and specific proportions are noted).<sup>7</sup>

**Table 6. Mean Annual Per Patient Societal and Health Care Sector Perspective Costs Related to DMD**

Health State	Health Care Sector Costs [95% CI]	Societal Perspective Costs [95% CI]	Source
Ambulatory	\$22,439 [\$18,178, \$29,312]	\$39,958 [\$32,367, \$52,197]	Landfeldt 2014
Non-Ambulatory	\$60,268 [\$53,927, \$83,026]	\$117,087 [\$96,028, \$147,845]	Landfeldt 2014

We will then stratify the mean per-patient annual cost of illness for both ambulatory and non-ambulatory into health care sector and societal perspective costs based on a detailed cost analysis of DMD.<sup>7</sup> Health care sector perspective costs include direct non-medication health care costs, costs of medications (these include numerous medication categories and are viewed in the model as not including the primary treatment costs), and covered costs of aids and devices. The societal perspective cost of illness includes all available cost categories. We will adjust each of the average costs by category by the ratio of total ambulatory costs to total non-ambulatory costs to get health state costs by categories. Table 7 shows the breakdown of costs by category adjusted to ambulatory and non-ambulatory and the corresponding totals by perspective.

**Table 7. Modeled 2018 Annual Supportive Care Costs by Perspective and Health State by Category**

Costs	Ambulatory	Non-Ambulatory	Source
Direct Medical (Non-Medication)	\$18,513 [\$15,104, \$24,090]	\$32,966 [\$26,895, \$42,899]	Landfeldt 2014
Medications	\$1,645 [\$1,287, \$2,270]	\$2,930 [\$2,291, \$4,042]	Landfeldt 2014
Aids and Devices	\$2,281 [\$1,787, \$2,952]	\$4,062 [\$3,181, \$5,256]	Landfeldt 2014
<b>Total Health Sector Costs</b>	<b>\$22,439 [\$18,178, \$29,312]</b>	<b>\$39,958 [\$32,367, \$52,197]</b>	Landfeldt 2014
Non-Medical Community Services	\$6,049 [\$4,632, \$8,352]	\$10,772 [\$8,249, \$14,872]	Landfeldt 2014
Informal Care	\$10,628 [\$9,586, \$11,868]	\$18,925 [\$17,071, \$21,133]	Landfeldt 2014
Indirect Cost of Illness	\$17,130 [\$14,697, \$19,650]	\$30,503 [\$26,172, \$34,990]	Landfeldt 2014
Out of Pocket for DMD-Related Home Alterations and Other Uncovered Equipment	\$4,022 [\$2,488, \$6,788]	\$7,162 [\$4,430, \$12,088]	Landfeldt 2014
<b>Total Societal Costs</b>	<b>\$60,268 [\$49,581, \$75,970]</b>	<b>\$107,320 [\$88,289, \$135,280]</b>	Landfeldt 2014

## 2.6 Model Outcomes

Model outcomes will include LYs gained, QALYs gained, and total costs for each intervention over a lifetime time horizon. We will also present a cost per extra year in ambulation. In addition, for deflazacort relative to prednisone, conditional on available evidence, we may include costs per adverse event such as cost per treatment induced weight gain avoided and cost per cataract avoided. Costs and all outcomes will also be reported by the health state to understand the relative contribution of the different states. All costs and other outcomes will be reported as discounted values, using a discount rate of 3% per annum (with undiscounted outcomes reported in an appendix).

## 2.7 Model Analysis

In the base case, cost-effectiveness will be estimated using projected incremental cost-effectiveness ratios in terms of QALYs as well as LYs and other outcomes, with incremental analyses comparing deflazacort to prednisone from a health care sector perspective and a modified societal perspective.

### Threshold Analyses

For treatments where there is insufficient evidence of a treatment effect but there are available prices (i.e., eteplirsen and golodirsen), the model will be used to project threshold treatment effects for the treatments to be considered cost-effective to achieve an incremental cost effectiveness ratio over a range of thresholds in terms of shifting the time to loss of ambulation and the time until death using “parallel shifts” of both survival curves in the model. Thresholds for drug costs across a range of incremental cost-effectiveness ratios from \$50,000 to \$500,000 per QALY (\$50,000, \$100,000, \$150,000, \$300,000, and \$500,000) will be estimated as DMD falls under ICER’s [ultra-rare disease framework](#).

### Sensitivity Analyses

We will conduct one-way sensitivity analyses to identify the impact of parameter uncertainty and key drivers of model outcomes. Probabilistic sensitivity analyses will also be performed by jointly varying all model parameters over 10,000 simulations, then calculating 95% credible range estimates for each model outcome based on the results.

### Scenario Analyses

Depending on available data, sensitivity analyses varying key assumptions will be explored.

## **Model Validation**

We will use several approaches to validate the model. First, we will present preliminary model methods to available manufacturers, clinicians, and patient groups, and subsequently draft methods and results to clinical experts who serve as external reviewers for this report. Based on feedback from these groups, we will refine data inputs used in the model, as needed. Second, we will vary model input parameters to evaluate face validity of changes in results. We will perform model verification by internal reviewers. As part of ICER's efforts in acknowledging modeling transparency, we will also share the model with available manufacturers for external verification around the time of publishing the draft report for this review. Finally, we will compare results to other cost-effectiveness models in this therapy area. The outputs from the model will be validated against the trial/study data of the interventions and also any relevant observational datasets.

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