

# Budget impact analysis of birch triterpenes for patients with epidermolysis bullosa from a United States perspective

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## Background

- Epidermolysis bullosa (EB) is a group of severe genetic conditions leading to increased skin fragility and chronic wounds.<sup>1,2</sup> There are 4 major categories, but those with junctional EB (JEB) and dystrophic EB (DEB) (including dominant dystrophic EB [DDEB] and recessive dystrophic EB [RDEB]) have more fragile skin than other EB subtypes, which may result in a more severe disease classification.<sup>1,2</sup>
- Wounds and wound care have a devastating impact on quality of life (QoL) for patients and caregivers, especially regarding dressing changes, which are extensive, frequent, time-consuming, and painful.<sup>1,2</sup> There is a significant financial burden associated with EB, where wound complications drive costs, especially with respect to healthcare resource utilization (HCRU) and wound care supplies.<sup>1,2</sup>
- There is no curative treatment for EB; established clinical management of EB is palliative in nature, focusing on dressings and wound management, reducing risk of new injury, minimizing complications, and improving QoL.<sup>1,2</sup>
- Filsuvez<sup>®</sup> (birch triterpenes; also known as birch bark extract) is a birch bark extract gel that received approval from the FDA in December 2023 for the treatment of wounds associated with DEB and JEB in adult and pediatric patients 6 months of age and older.<sup>1</sup>

## Objective

- The objective of this study was to evaluate the potential economic implications of adding birch triterpenes to routine standard of care (SoC) for the treatment of adult patients with EB from a United States (US) third-party payer perspective.

## Methods

### Model overview

- A Microsoft Excel-based budget impact analysis was developed to estimate total drug and healthcare expenditures and the incremental budget impact of introducing birch triterpenes as an add-on to routine care in adult or pediatric patients with JEB or DEB (**Figure 1**).
- Incremental costs were calculated as the difference between the market with birch triterpenes included and the market without birch triterpenes (ie, SoC). Costs in the model included pharmacy and HCRU costs.
- The base case considered a mix of payers (commercial, Medicare, and Medicaid) and covered medical costs of adding birch triterpenes to routine care in patients with DEB or JEB over a 3-year time horizon (**Table 1**).

### Model inputs and data sources

- Model inputs were sourced from literature and clinical trial data, with costs adjusted to 2024 US dollars (USD). Costs included in the model were drug acquisition/administration, bandages, home healthcare, and other HCRU costs (**Table 1**, **Table 2**).
- Results are estimated based on an assumed plan population of 1,000,000 members. Market share for birch triterpenes was expected to increase from 12.9% to 32.4% over 5 years for JEB, 10.6% to 27% for DDEB, and 17.9% to 57.8% for RDEB. The model assumed an annual treatment discontinuation of 5%.
- The modeling approach, inputs, and assumptions were validated by a US clinician and 2 US ex-payer experts.

Table 1. Drug acquisition and bandage costs			
Parameter	Birch triterpenes	SoC	Source
Drug inputs			
Acquisition cost per month (WAC)	\$1,800	N/A	Merative Micromedex 2024 <sup>3</sup>
Number of tubes per month	27	N/A	Mean usage in EASE trial <sup>4</sup>
Bandage cost inputs			
Mean dressing changes per week	4.7	5.2	Bruckner 2024 <sup>5</sup>
Annual cost of bandages (reimbursed only, not including OOP)			
JEB	\$479.04	\$530.00	Feinstein 2022 <sup>6</sup>
DDEB	\$118.40	\$131.00	Feinstein 2022 <sup>6</sup>
RDEB	\$4,827.44	\$5,341.00	Feinstein 2022 <sup>6</sup>

Key: DDEB – dominant dystrophic epidermolysis bullosa; JEB – junctional epidermolysis bullosa; N/A – not applicable; OOP – out of pocket; RDEB – recessive dystrophic epidermolysis bullosa; SoC – standard of care; WAC – wholesale acquisition costs. Note: Additional healthcare resource utilization costs pertain to ambulatory visits per year,<sup>7,8</sup> emergency department visits per year,<sup>7,8</sup> inpatient visits per year,<sup>7,8</sup> and annual medications.<sup>9</sup> Annual costs of bandages for birch triterpenes are estimated by applying the proportional difference in dressing changes per week to the annual cost for SoC patients.

### Scenario analyses

- Scenario analyses with varying cost inputs were utilized to assess the impact of improved wound healing with birch triterpenes, productivity savings via decreased caregiver time with birch triterpenes, and inclusion of out-of-pocket bandage costs.
  - Emergency department and medication use HCRU inputs were reduced by 13% based on the improved wound healing for those treated with birch triterpenes observed in the EASE clinical trial (50.5% improvement in wound healing vs 43.9% for SoC after 2 years).<sup>4</sup>
  - Based on estimated weekly dressing changes, caregivers spend an average of 4.4 hours dressing wounds with SoC compared to only 1.6 hours for patients treated with birch triterpenes.<sup>11</sup> Based on a mean hourly wage of \$14.87 for home health professionals, this equated to a total annual cost model input of \$3,402 for SoC compared to \$1,237 for birch triterpenes.
  - Out-of-pocket bandage costs vary based on the type of EB.<sup>12</sup> For SoC, out-of-pocket input expenses for JEB, DDEB, and RDEB were \$9,480, \$7,680, and \$8,800, respectively.<sup>12</sup> Based on data collected in the EASE study, out-of-pocket inputs for patients treated with birch triterpenes were estimated by decreasing SoC inputs by 9.6% (mean number of weekly bandage/dressing changes was 4.7 for birch triterpenes patients vs 5.2 for SoC).<sup>5</sup>

Table 2. Total healthcare resource use costs				
	Commercial	Medicare	Medicaid	Other payer
Payer mix percentage	36%	38%	26%	N/A
Commercial to noncommercial ratio	N/A	188%	261%	261%
JEB	\$38,330	\$20,388	\$14,679	\$14,679
DDEB	\$36,898	\$19,627	\$14,131	\$14,131
RDEB	\$42,751	\$22,740	\$16,373	\$16,373

Key: DDEB – dominant dystrophic epidermolysis bullosa; JEB – junctional epidermolysis bullosa; N/A – not applicable; RDEB – recessive dystrophic epidermolysis bullosa. Note: Total healthcare resource use includes annual costs for medical, home health (eg, nursing services provided in the home, wound dressings, associated supplies), and pharmacy.<sup>9-10</sup> Costs are taken from an analysis of claims data for patients diagnosed with EB and inflated to 2024 costs. These costs are assumed to be for commercially treated patients; as such, Medicare, Medicaid, and other payer costs are adjusted using national commercial to noncommercial ratios.<sup>9,10</sup>

Table 3. Budget impact over 3 years

	Current market (without birch triterpenes)	New market (with birch triterpenes)	Incremental difference
Number of patients who initiated birch triterpenes	0	1.36	1.36
Costs			
Drug acquisition	\$0	\$1,758,806	\$1,758,806
Bandages	\$23,170	\$22,204	−\$966
Home healthcare	\$33,492	\$33,492	\$0
Other HCRU	\$229,560	\$229,560	\$0
Total cost	\$286,221	\$2,044,061	\$1,757,840
Cost PMPM			\$0.05

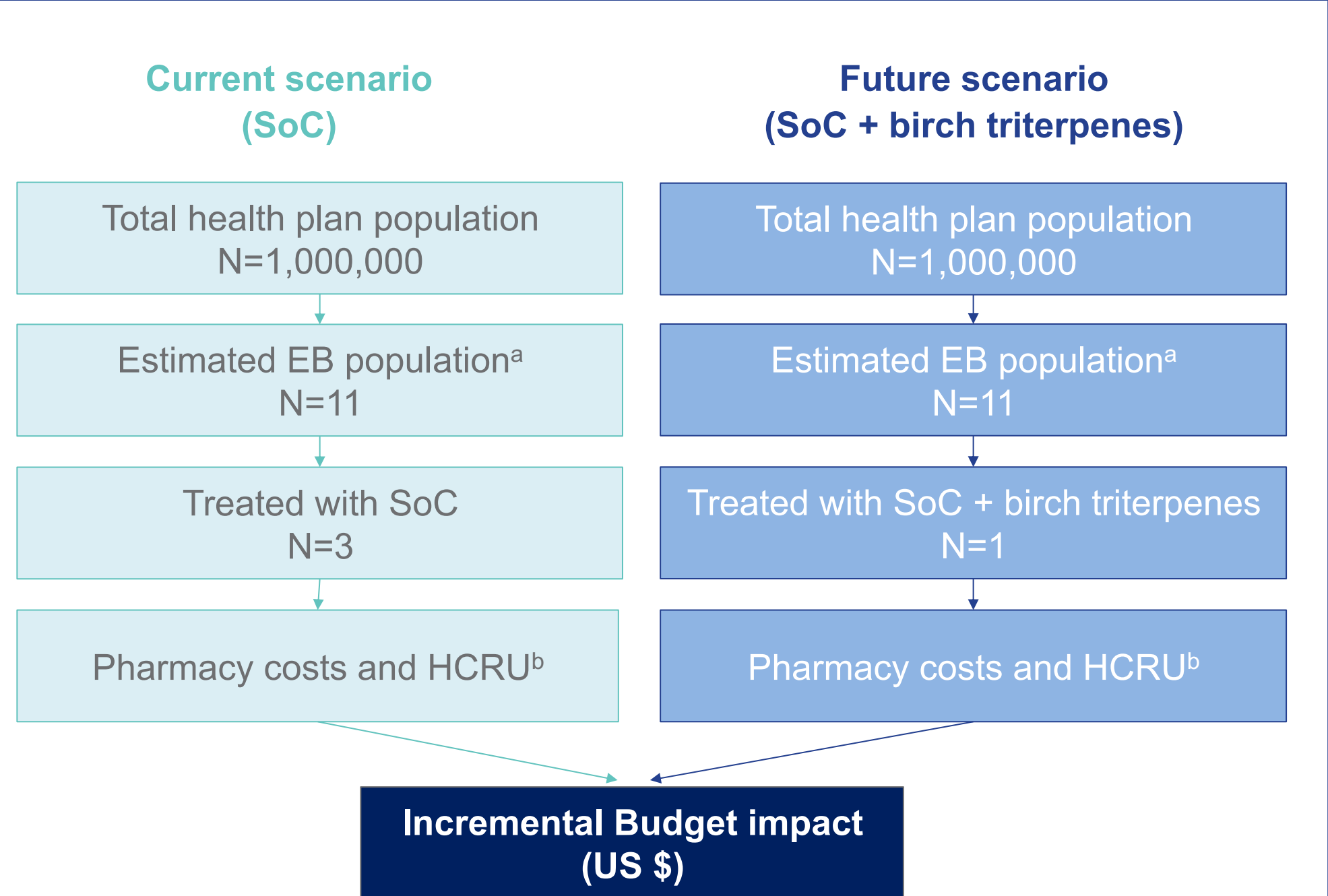
Key: HCRU – healthcare resource utilization; PMPM – per member per month.

Table 4. Scenario analysis

	Current market (without birch triterpenes)	New market (with birch triterpenes)	3-year cumulative budget impact	Difference from base case
Base case	\$286,221	\$2,044,061	\$1,757,840	-
Improved wound healing <sup>a</sup>	\$276,281	\$2,029,360	\$1,753,078	−\$4,762
Caregiver productivity savings	\$286,221	\$2,031,550	\$1,745,329	−\$12,511
Include out-of-pocket bandage costs	\$370,774	\$2,126,008	\$1,755,234	−\$2,606

<sup>a</sup>This scenario must be paired with the micro-costing HCRU model inputs as this function proportionally reduces costs relating to emergency department visits and medication use, but not inpatient or ambulatory visits. Key: HCRU – healthcare resource utilization; SoC – standard of care.

Figure 1. Budget impact model approach with/without birch triterpenes



Key: DDEB – dominant dystrophic epidermolysis bullosa; HCRU – healthcare resource utilization; JEB – junctional epidermolysis bullosa; RDEB – recessive dystrophic epidermolysis bullosa; SoC – standard of care. <sup>a</sup>Values used in model are calibrated to estimates of 0.49 per million (JEB), 1.49 per million (DDEB), and 1.35 per million (RDEB), as published in Fine 2016.<sup>2</sup> <sup>b</sup>HCRU included costs for bandages, ambulatory visits, inpatient stays, medication, and accident and emergency visits.

Figure 3. Total budget impact with/without birch triterpenes

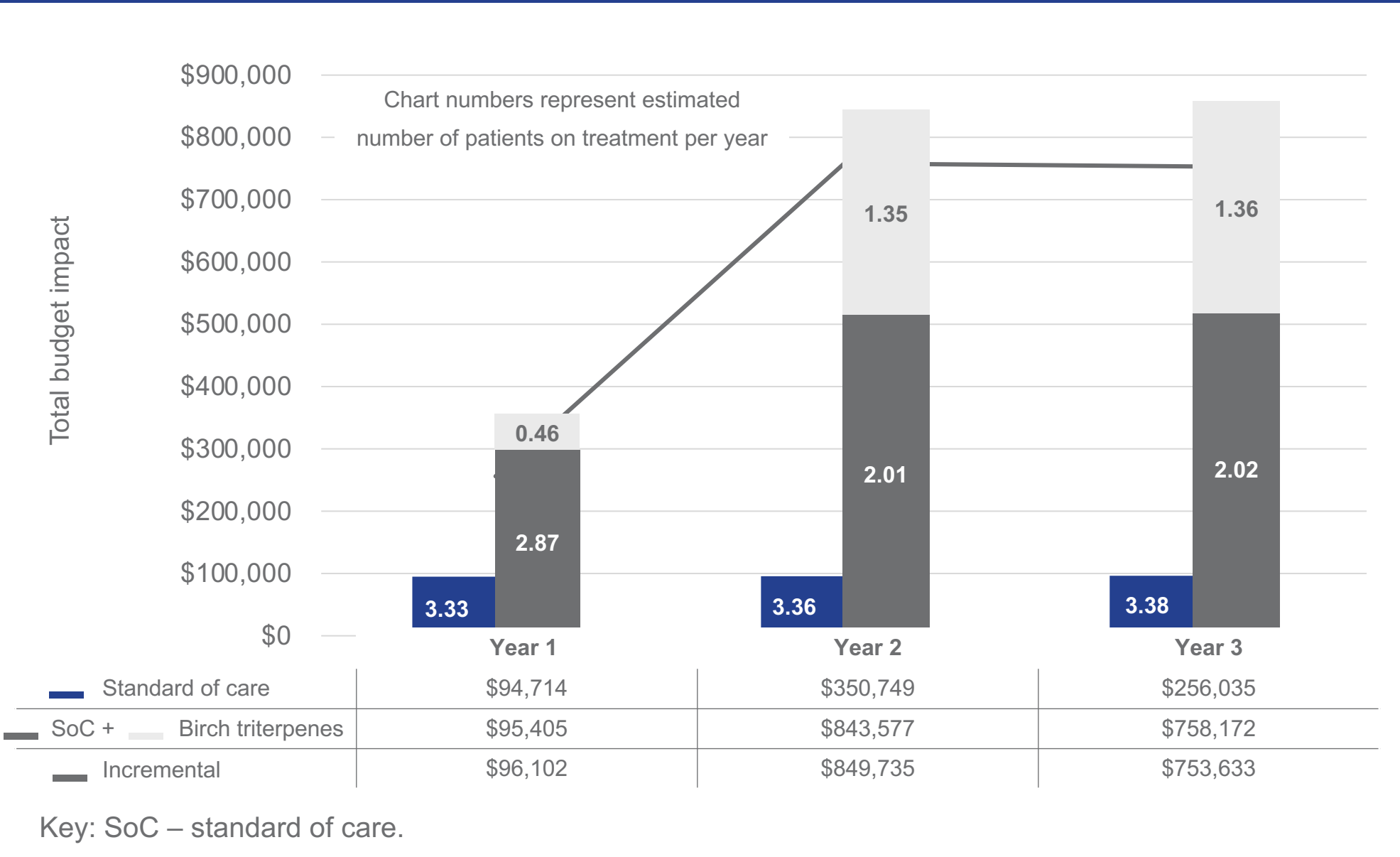
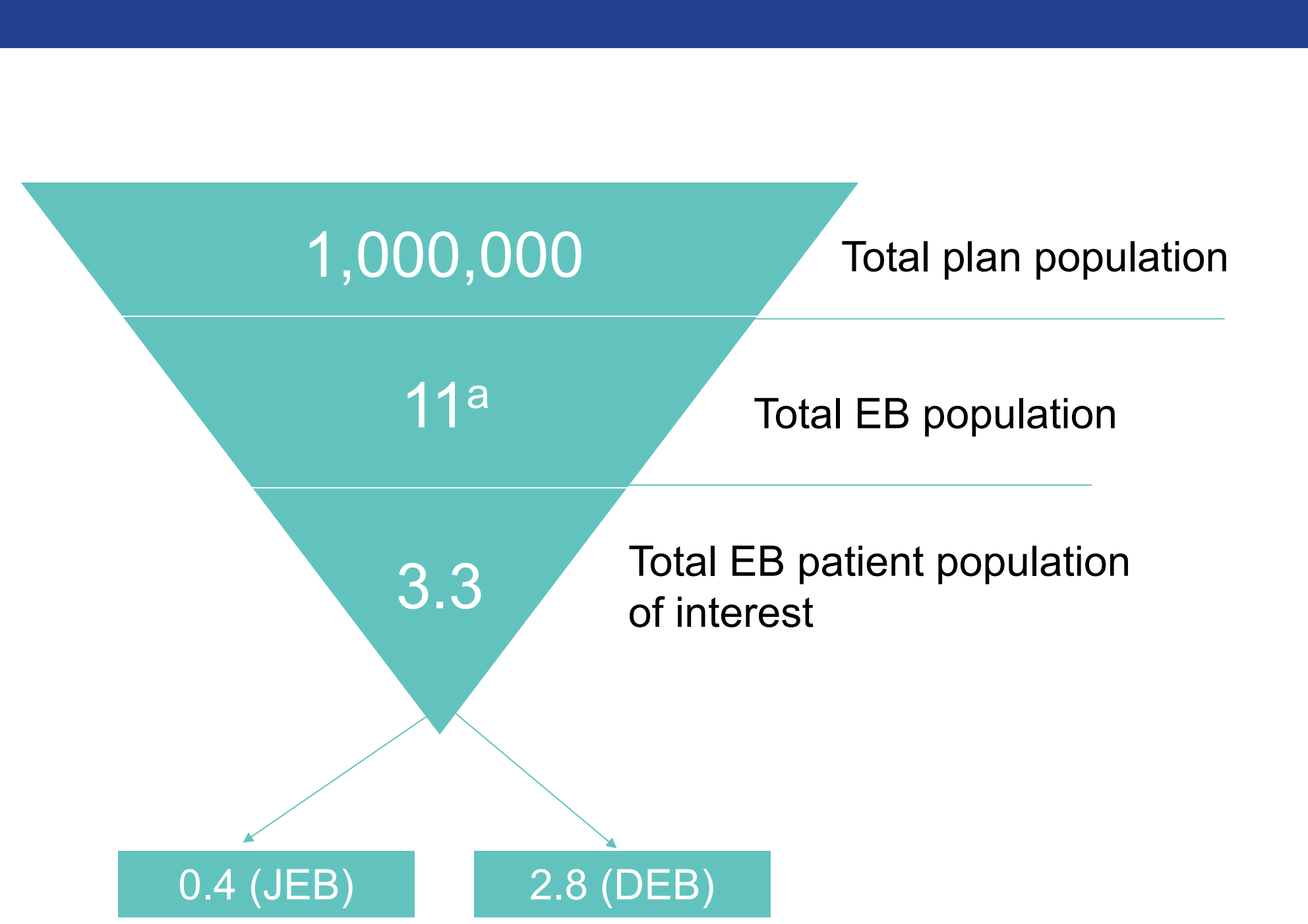
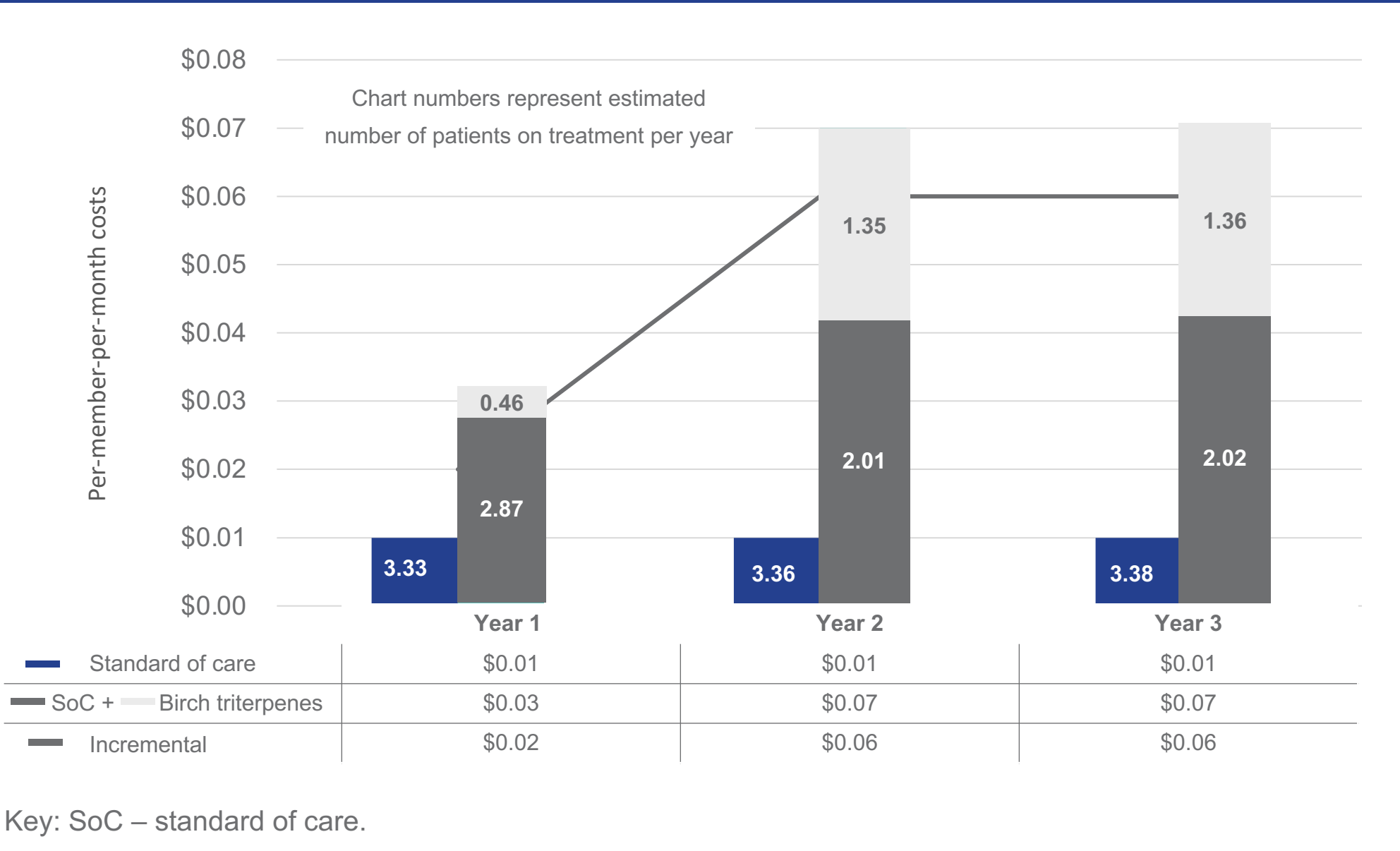


Figure 2. Plan population: Number of patients



Key: DEB – dystrophic epidermolysis bullosa; EB – epidermolysis bullosa; JEB – junctional epidermolysis bullosa. <sup>a</sup>Values used in model were based on EB subtype population estimates as published in Fine 2016.<sup>2</sup>

Figure 4. Per-member-per-month costs



## Results

### Base case results

- In a plan of 1 million lives, there were an estimated 0.4 patients with JEB and 2.8 patients with DEB. In the base case with estimated market shares, only **1.36 patients per plan** were estimated to receive birch triterpenes alongside SoC (**Figure 2**).
- Adding birch triterpenes to routine care increased the 3-year total plan costs by an estimated \$1,757,840 (**Figure 3**), with a **cost per member per month of \$0.05** (**Figure 4**).
- Total 3-year costs before and after the introduction of birch triterpenes were estimated at \$286,221 and \$2,044,061, respectively (**Table 3**).

## Sensitivity analysis

- The one-way sensitivity analysis varied base case inputs by ±10% to show which inputs had the biggest impact on the results.
- The model was most sensitive to variation in the average number of birch triterpenes tubes per month and the plan population, while it was less sensitive to DDEB and JEB patient prevalence (**Figure 5**).

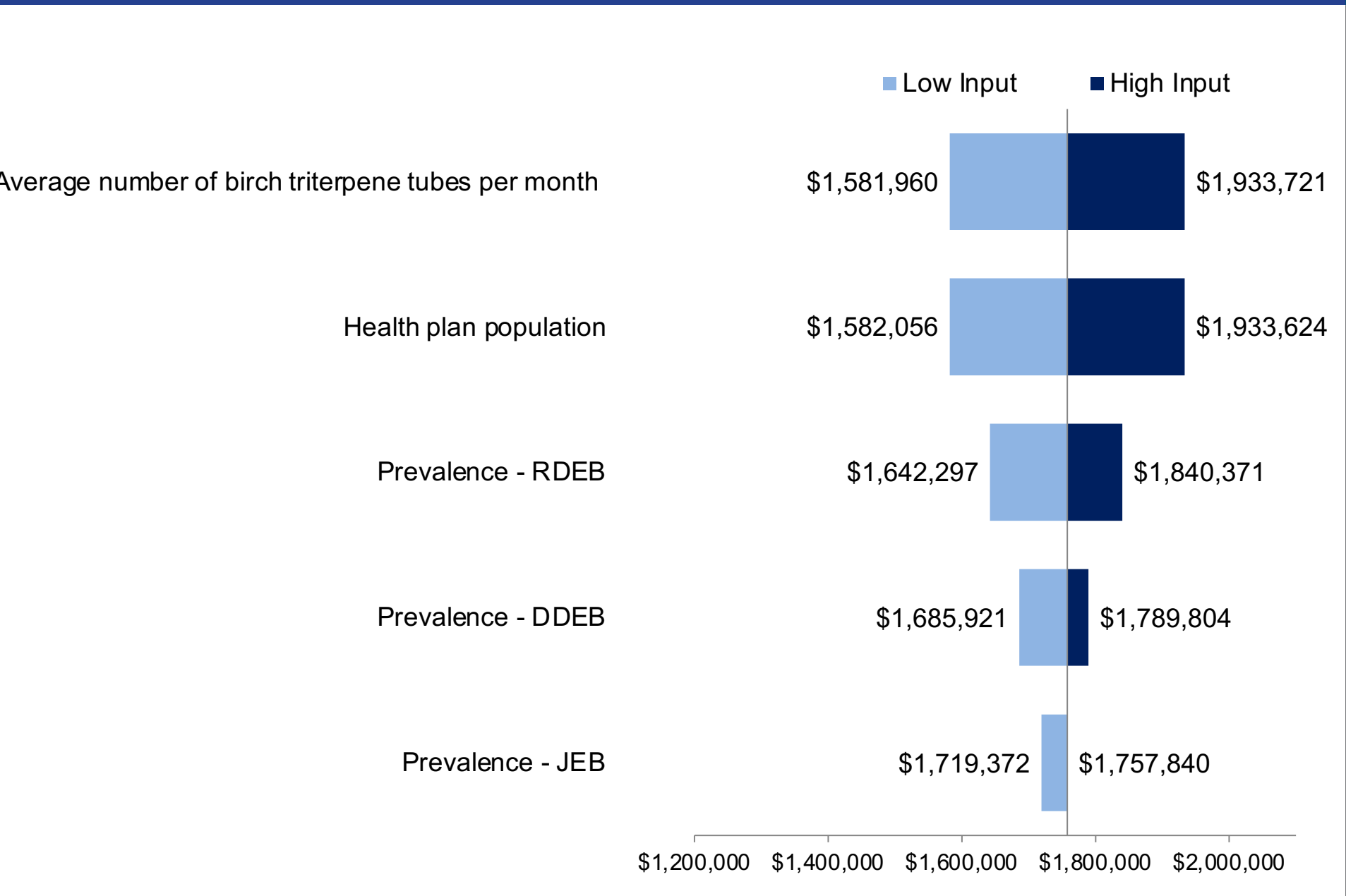
## Scenario analyses

- Scenario analyses resulted in decreased cumulative 3-year budget impacts with the addition of birch triterpenes to SoC (ie, without birch triterpenes) compared to the base case (**Table 4**).

## Limitations

- Models represent a simplification of complex factors in the healthcare system. Estimated costs could vary.
- EB is a complex and ultra-rare genetic disorder. The included cost data (presented in 2024 USD) are based on limited published sources. Costs are associated with some uncertainty and may not be generalizable to all health systems.

Figure 5. One-way sensitivity analysis (+/- 10%) results



Key: DDEB – dominant dystrophic epidermolysis bullosa; JEB – junctional epidermolysis bullosa; RDEB – recessive dystrophic epidermolysis bullosa.

**References:** 1. Filsuvez. Prescribing information. Chiesi, Inc; 2024. 2. Fine JD. *JAMA Dermatol*. Nov 1 2016;152(11):1231-1238. 3. Merative Micromedex. Red Book prices. Accessed June 2024. <https://www.micromedexsolutions.com/micromedex2/librarian/PFDDefaultActionId/redbook/ModifyRedBookSearch/ssl/true> 4. Kern JS, et al. *Br J Dermatol*. Jan 23 2023;188(1):12-21. doi:10.1093/bjd/ljac001 5. Bruckner A, et al. *J Invest Dermatol*. 2024;144(6):S47. 6. Feinstein J, et al. *Orphanet J Rare Dis*. 2022;17:367. 7. CMS. Physician Fee Schedule. Accessed June 2024. <https://www.cms.gov/medicare/payment/fee-schedules/physician> 8. AHRQ. Healthcare Cost and Utilization Project (HCUPnet). Accessed June 2024. <https://dataools.ahrq.gov> 9. Anderson C, et al. Commercial reimbursement benchmarking. Accessed June 2024. <https://www.milliman.com/en/insight/commercial-reimbursement-benchmarking-payment-rates-medicare-fee-for-service> 10. KFF. Medicaid-to-Medicare Fee Index. Accessed June 2024. <https://www.kff.org/medicaid/state-indicator/medicaid-to-medicare-fee-index/?currentTimeframe=0&sortModel=%7B%22colId%22:%22Location%22,%22sort%22:%22asc%22%7D> 11. Kirtisi D. Oleogel-S10 reduces dressing changes burden in patients with epidermolysis bullosa. Presented at: EADV Congress; October 11-14, 2023; Berlin, Germany. 12. Gorell ES, et al. *Pediatr Dermatol*. Nov 2020;37(6):1198-1201.

## Conclusions

- EB is an ultra-rare and debilitating disease that affects QoL for patients and caregivers, which may result in a substantial socioeconomic burden.
- In the base case plan of 1 million lives and projected market shares, adding birch triterpenes to SoC results in a cumulative increase in total 3-year plan costs by an estimated \$1,757,840, with a cost per member per month of \$0.05.
- Scenario analyses, including out-of-pocket costs and caregiver productivity, reflect the high, direct nonhealthcare costs and use of informal care needs of EB that can result in considerable societal costs.
- Introduction of birch triterpenes as a new treatment for wounds associated with DEB and JEB alongside routine care has a manageable budget impact in a 3-year plan.

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