APPENDICES

Voxelotor (Oxbryta) for Sickle Cell Disease

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The Medicaid Evidence and Review of Cost Initiative (MERCI) describes policy considerations for drugs approved by the US Food and Drug Administration (FDA) through the accelerated approval pathway. This document is the appendix of a brief entitled *Voxelotor (Oxbryta) for Sickle Cell Disease*. The brief and the associated appendix provide information on: the estimated prevalence of target diagnoses (the accelerated approval drug's indication[s]) among Medicaid members; the clinical trial population used to support FDA approval, and how similar it is to Medicaid members overall; and projected drug costs for state Medicaid programs, including a breakdown of state and federal funds using the Federal Medical Assistance Percentage (FMAP).

APPENDIX A

METHODS

Data Sources

Researchers from the Center for Evidence-based Policy (Center) used the Transformed Medicaid Statistical Information System (T-MSIS) Analytic Files (TAF) as the primary data source for drug indication cohort identification, prevalence estimates, and medication uptake. The TAF are a research-optimized version of state-submitted T-MSIS data, which include information on Medicaid and Children's Health Insurance Program (CHIP) enrollment, demographics, health care service use, and payments, based on enrollment and claims records. State-submitted T-MSIS data are processed by the University of Minnesota Research Data Center, and then compiled for use as national data files.

We obtained TAF demographic and enrollment data, with inpatient, other services, and pharmacy claims data for years 2019 through 2021 for all Medicaid and CHIP members aged 0 to 64, excluding those with any months of dual enrollment in Medicaid and Medicare. Using these criteria, we were not able to obtain data from Utah or Alabama, as these states do not submit claim information related to dual enrollment status using this method. Cohorts for analysis were anchored in the most recent year of data available (2021), with preceding years used to maintain internal validity for diagnosis and service-use identification, based on established methods specific to the indication of interest. Other sources that informed cohort definitions, drug indication, and drug identification included peer-reviewed literature, grey literature sources, and publicly available databases.

The TAF data are subject to quality concerns. To identify data quality or usability issues affecting internal analytical validity, the Medicaid Data Quality (DQ) Atlas was used as a reference.[1] In general, if the DQ Atlas identified a state's data as "unusable" for a topic, variable, or year, that state was eliminated from analysis. If a state's data were of "high concern," we investigated further to determine the reason behind the rating and made a topic-specific or variable-specific judgment, to determine inclusion or exclusion for analysis; we made decisions to include, with a bias towards underreporting (as opposed to overreporting). We used 3 distinct methods to address large-scale data quality issues during initial data processing, as described below.

Member Demographic Identification and State Assignment

Members have 2 identifiers in the TAF: a primary identifier assigned during processing at University of Minnesota Research Data Center, which compiles claims across states for individual members, and a member-specific identifier (MSIS ID) assigned by the state (plus the identifying state). Ninety-seven percent of members had primary identifiers. For the remaining 3%, we used the combination of MSIS ID and state code. A very small proportion of members with primary identifiers had multiple enrollment records, sometimes with differing state codes and demographic information. Those members were assigned a state code based on the highest frequency and consistency of the following attributes, in order: state of residence, state with the highest proportion of claims, and state with the

longest period of enrollment. If there were ties among states for a member, we randomly assigned them to 1 of the states within which they had claims.

Differences in demographic information for members with multiple enrollment records were similarly reconciled. In the case of multiple records with missing demographic information, missing values were imputed from records assigned to the member in other states, or the most frequently reported characteristic was assigned. Race and ethnicity were the most common missing characteristic; age and sex were rarely missing in this dataset.

Mississippi Member Identification and Claims

Data linking of Mississippi claims records to member enrollment records was considered "unusable" by the DQ Atlas for 2019 to 2021. Any members with claims submitted in Mississippi were assigned to that state for drug indication prevalence reporting. The only demographic information that we could identify for members from Mississippi was birth date from submitted claims. We could not use sex, race, or ethnicity information in the enrollment files for these members. In the brief, only the following data are included from Mississippi:

- Number of people with drug indication, if no demographic information other than age is required for cohort inclusion
- A breakdown of members with a particular drug indication by age (sample size permitting)
- Comorbidities and health care service use for members with the drug indication, and matched comparisons where matching is based only on age
- Drug uptake, if applicable

In the case that other demographic characteristics are required for cohort inclusion (e.g., sex), members from Mississippi were not included.

Illinois Claims

Illinois claims data are known to be reported with multiple records per care episode, or "claim families," which would otherwise be aggregated into a single claim record in other states. Methods for including Illinois claims were applied according to TAF Technical Guidance resources and recommendations.[2]

Reporting of Data

Adhering to CMS reporting rules, we reported member counts in any subgroup only when the group size was at least 11. We reported rates and percentages when the group size on the numerator was at least 11 and the denominator group size was at least 50. For example, demographic groups, rates of health care service use, and drug uptake were not reported for states with fewer than 50 Medicaid members with SCD. Similarly, if there were any race or ethnicity groups with 10 or fewer people, then only the largest group was reported when total of the unreported group sizes was greater than 10.

Prevalence Estimates

To identify members with sickle cell disease (SCD), we used a claims-based, case-finding algorithm with a 3-year lookback period.[3] A member was classified as having SCD if at least 1 inpatient claim or 2 other service claims on separate days with ICD-10 (International Classification of Diseases, 10th revision) diagnosis code prefix D57 (except D57.3 for sickle cell trait). This algorithm has been validated with high accuracy against screening data for pediatric Medicaid patients[3] and used in several other studies[4-7] to identify SCD patients in claims data. The cohort was further restricted to ages 4 through 64 in accordance with the drug indication. To identify drug uptake, we searched prescription files for claim lines containing National Drug Code (NDC) directory codes *72786-111-02*, *72786-111-03*, *72786-101-01*, *72786-102-02*, and *72786-102-03*, and then linked them by member identifier.[8] We only identified NDC code 72786-101-01, approved in 2019, in the data.

Matched Comparison Group

We used a matched-comparison method to analyze health care service use and health states between members with the drug indication and the Medicaid population at large. We performed 1-to-3 exact matching between members with SCD and members without SCD, based on member state, sex, age in years, and race/ethnicity group, when available. If we identified more than 3 exact matches for a member with SCD, 3 were chosen at random.

Comorbid Conditions

We used the Chronic Disability Payment System (CDPS) algorithm to identify the prevalence of affected body systems and relevant comorbidities in the SCD cohort and matched comparisons.[9] The CDPS has a hierarchical method to classify members into risk groups by body system using ICD-10 diagnosis codes in medical claims. There are multiple risk groups per body system, and a member may only belong to 1 risk group per body system. Once categorized, we aggregated risk groups into whole-system categories (e.g., cardiovascular, pulmonary). We then identified and searched for chronic conditions specific to the population with the drug indication in the data using the CMS Chronic Conditions Warehouse algorithm definitions and methods.

Health Care Service Use

We compared 2 health care service use outcomes (hospitalizations and emergency department [ED] visits), measured in both the drug indication and matched comparison groups, between January 1, 2021 and December 31, 2021. We identified hospitalizations in the inpatient files as episodes of care based on unique admission date. Unique discharge dates were used in the case of missing admission dates. We identified ED visits in both inpatient and outpatient files using revenue center codes 450 through 459 and 981, and service date. Accordingly, the ED visits we report include those that resulted in an admission.

Medication Adherence and Uptake

Medication uptake was calculated as the proportion of unique members with SCD with any identified claim in the prescription files for voxelotor (see above). Medication adherence was calculated using the Medication Possession Ratio (MPR), a member-level estimation calculated as the proportion of days' supply provided during a given time period, for the year 2021. The numerator is the number of days' supply obtained, starting at the date of the first fill of the year, and the denominator is the total number of days between the first fill and December 31, 2021. This method assumes the medication is prescribed for daily, continuous use, and that the member took their medication as prescribed.

Cost Estimates

The cost estimates represent the projected annual total national costs associated with covering voxelotor for treatment of Medicaid members with SCD, under different uptake and adherence scenarios. We modeled the costs based on the drug indication prevalence and drug uptake and adherence observed in the TAF Medicaid claims data, as well as current wholesale acquisition cost (WAC) for the drug and statutorily required rebate percentages. Voxelotor is available in 3 dosage forms and strengths and 2 packaging sizes, as described in Exhibit A1. The per-tablet prices for each dosage form and packaging size are chosen in a way that equates the annual cost of each option. The other model inputs and justifications are summarized in Exhibit A2.

EXHIBIT A1

Voxelotor dosage forms and packaging options and prices

Dosage form (strength)	Package size, days	2019 to 2021 annual price, $	Current annual price[b], $
Oral tablet (500 mg)	90	126,740	147,389
Oral tablet (300 mg)	60	*n/a*[a]	147,389
Oral tablet (300 mg)	90	*n/a*[a]	147,389
Tablet for oral suspension (300 mg)	60	*n/a*[a]	147,389
Tablet for oral suspension (300 mg)	90	*n/a*[a]	147,389

Note. [a] The 300-mg dosages of voxelotor (oral tablet, tablet for oral suspension) were not available until 2022.[10] [b] Costs are annualized based on patients taking the recommended daily dose and the packaging option providing 30-day supply at the recommended dose.
Abbreviation. n/a: not applicable.

EXHIBIT A2

Cost modeling inputs

Input name	Input	Source	Sensitivity analysis bounds	Justification
Prevalence/uptake[a]				
Prevalence (# members with SCD)	47,140	Data	42,126 to 52,551	Lower bound: correcting for data quality issues (overreporting in MA and NJ) and using a stricter definition of SCD (having at least 3 claims with SCD diagnosis and considering only the first 2 diagnosis codes in inpatient claims). Upper bound: correcting for data quality issues (underreporting in RI) and using a less strict definition of SCD (having any claim with SCD diagnosis code).
Uptake (% patients with SCD using voxelotor)	15%	Data	5% to 30%	Lower bound: for 2021, voxelotor uptake in Medicaid data was 5.4%. Newman et al. (2023) found 4.6% uptake rate for voxelotor among individuals with private insurance.[11] Upper bound: Newman et al. (2023) found uptake rate for any disease modifying treatment was 28.3% among patients with a commercial insurance in 2021.[11]
Drug cost				
Annual drug cost (WAC)	$147,389	IPD Analytics	--	--
Federal rebates[b]	23.1%	SSA §1927(c)(1)(B)(i)[12]	--	--
Adherence				
Adherence, %	35%	Data	25% to 70%	Discontinuation in the trials was 27.3%, all reasons combined.[13]
Average days' supply (adherent)	310	Data	203 to 365	Input: average supply was 310 in 2021 for adherent patients with access to the drug (i.e., ≥ 1 claim for voxelotor) in 2020. Lower bound: average supply in 2021 for all adherent patients.
Average days' supply (nonadherent)	105	Data	Based on the distribution and parameters observed in the data.	

Notes. [a] Alabama and Utah included in cost estimates based on number of Medicaid members with SCD reported for these states.[14] [b] Do not include state-negotiated supplemental rebates.

Abbreviations. MA: Massachusetts; NJ: New Jersey; RI: Rhode Island; SCD: sickle cell disease; SSA: Social Security Administration; WAC: wholesale acquisition cost.

As our focus is direct drug costs, we did not include the costs of drug dispensing and monitoring. Due to lack of published data, we also did not include cost offsets associated with replacement of treatment-as-usual. Similarly, we did not include cost implications of treatment effectiveness in terms of recovery, reduced health care service use, or mortality.

We performed sensitivity analyses using Monte Carlo simulations, taking into consideration uncertainty in the model inputs, and reporting the range that contained 95% of the simulated cost values as the confidence bounds for our cost estimate. We also performed 2-way scenario analyses to show how the cost estimate changes under different uptake and adherence scenarios. We then calculated the most likely cost estimates with the 95% confidence bounds for each state separately, using the estimated drug indication prevalence for each state given in Exhibit B, and the upper and lower bounds for prevalence calculated using the same method described in Exhibit A2. For all other model inputs, we used the national cost estimate.

For our per-member per-month (PMPM) cost estimates, nationally and for each state, we used the member month counts we observed in the 2021 data, excluding any dually enrolled members. For the state and federal breakdown of the costs in each state, we first calculated the percentage of the members with SCD in CHIP and adult Medicaid Expansion enrollment categories. We then applied the corresponding federal matching rates in each state to the relevant portion of the total costs using the current Federal Medical Assistance Percentage (FMAP) rates[14,15] for the portion of the costs by Medicaid and CHIP members and applied the 90% FMAP exception[16] for the portion of the costs by the members with adult Medicaid Expansion enrollment. For states with unusable data quality for identifying CHIP enrollment, we used the average percentage of CHIP enrollment in other states. Similarly, for expansion states with unusable data quality for identifying Medicaid adult expansion enrollment, we used the average of adult expansion enrollment share in other expansion states. For the state and federal breakdown of the costs nationally, we used the average state FMAP and enhanced FMAP rates for Medicaid and CHIP groups.

The drug spending estimates for 2019 through 2021 are based on the actual days' supply in voxelotor pharmacy claims in 2019 through 2021. We used the drug WAC price and FMAP rates in the corresponding years, rather than the current prices and rates.

Although we were able to report cost estimates at the state level only for states with more than 50 SCD patients eligible for the drug, our national cost estimates include all patients in all states, including those without data on all cost inputs. In all cost calculations for states with any missing input, we used estimates for those inputs from the literature and from other states. Specifically, for the missing drug indication prevalence in Alabama and Utah, we used the SCD prevalence rates among Medicaid members in these states reported elsewhere.[14] We applied population percentages reported by Census Bureau for the younger than age 4 group in these states to identify the SCD patient population

eligible for voxelotor. The member month counts in Alabama used in PMPM cost estimates for this state were based on monthly Medicaid enrollment data reported.[17]

Limitations

Our cost estimates are based on the prevalence of sickle cell disease and voxelotor use patterns we identified in the claims data. Given that the T-MSIS TAF do not include clinical information relevant to identifying individuals eligible for voxelotor, we had to approximate the clinical indication using a claims data-based approach. As such, the accuracy of our analysis depends on the completeness and reliability of the claims and the information recorded in the data (e.g., diagnosis and procedure codes in the inpatient and outpatient claims, NDC codes, and days' supply information in pharmacy claims) as well as enrollment and demographic information (e.g., dual enrollment, age) for each member.

For the 2 states without claims data, our cost estimates rely on the prevalence of the condition reported elsewhere and the assumption that voxelotor use patterns in these states are similar to what is observed in other states. Our cost estimates do not include supplemental rebates and the estimated total cost is broken down by state and federal share without any consideration for third-party liability or other insurance payments.

APPENDIX B
MEDICAID MEMBERS WITH AND WITHOUT SCD, 2021

EXHIBIT B

Medicaid members aged 4 to 64 with and without sickle cell disease, 2021

		Members with SCD			Members without SCD
State	Total Medicaid population	n	Per 10,000 members	%	n
United States	*73,930,771*	*45,625*	*6.2*	*0.06*	*73,885,146*
Alabama[a]	--	--	--	--	--
Alaska	211,534	16	0.8	0.01	211,518
Arizona	1,884,893	415	2.2	0.02	1,884,478
Arkansas	878,989	569	6.5	0.06	878,420
California	13,050,054	2,606	2.0	0.02	13,047,448
Colorado	1,373,458	220	1.6	0.02	1,373,238
Connecticut	894,814	598	6.7	0.07	894,216
Delaware	240,436	268	11.1	0.11	240,168
District of Columbia	224,502	379	16.9	0.17	224,123
Florida	3,423,157	4,194	12.3	0.12	3,418,963
Georgia	1,936,824	3,182	16.4	0.16	1,933,642
Hawaii	358,322	16	0.4	0.00	358,306
Idaho	358,743	23	0.6	0.01	358,720
Illinois	2,900,097	2,107	7.3	0.07	2,897,990
Indiana	1,569,417	738	4.7	0.05	1,568,679
Iowa	675,511	225	3.3	0.03	675,286
Kansas	361,940	147	4.1	0.04	361,793
Kentucky	1,477,172	380	2.6	0.03	1,476,792
Louisiana	1,534,368	1,986	12.9	0.13	1,532,382
Maine	304,470	38	1.2	0.01	304,432
Maryland	1,364,653	1,845	13.5	0.14	1,362,808
Massachusetts	1,666,141	930	5.6	0.06	1,665,211
Michigan	2,453,513	1,546	6.3	0.06	2,451,967
Minnesota	1,064,915	431	4.0	0.04	1,064,484
Mississippi	518,370	1,058	20.4	0.20	517,312
Missouri	945,272	698	7.4	0.07	944,574
Montana[b]	256,520	--	--	--	--
Nebraska	278,831	109	3.9	0.04	278,722
Nevada	711,921	418	5.9	0.06	711,503
New Hampshire	207,662	23	1.1	0.01	207,639
New Jersey	1,701,560	1,388	8.2	0.08	1,700,172

State	Total Medicaid population	Members with SCD			Members without SCD
		n	Per 10,000 members	%	n
New Mexico	791,534	40	0.5	0.01	791,494
New York	5,777,465	4,613	8.0	0.08	5,772,852
North Carolina	2,121,187	2,002	9.4	0.09	2,119,185
North Dakota	95,578	13	1.4	0.01	95,565
Ohio	2,645,732	1,831	6.9	0.07	2,643,901
Oklahoma	921,201	290	3.1	0.03	920,911
Oregon	1,158,584	94	0.8	0.01	1,158,490
Pennsylvania	2,909,710	2,219	7.6	0.08	2,907,491
Rhode Island	292,582	148	5.1	0.05	292,434
South Carolina	1,113,901	1,493	13.4	0.13	1,112,408
South Dakota[b]	104,905	--	--	--	--
Tennessee	1,339,160	1,062	7.9	0.08	1,338,098
Texas	4,622,931	3,051	6.6	0.07	4,619,880
Utah[a]	--	--	--	--	--
Vermont	148,757	11	0.7	0.01	148,746
Virginia	1,576,987	1,291	8.2	0.08	1,575,696
Washington	1,782,948	287	1.6	0.02	1,782,661
West Virginia	519,918	45	0.9	0.01	519,873
Wisconsin	1,122,464	573	5.1	0.05	1,121,891
Wyoming[b]	57,168	--	--	--	--

Notes. [a] Data not available. [b] Data suppressed (N < 11).

APPENDIX C

DEMOGRAPHIC INFORMATION

EXHIBIT C1

Availability of demographic information for Medicaid members aged 4 to 64 years, 2021

	Medicaid members with SCD	%	Medicaid members without SCD	%
Total	*45,625*		*73,885,146*	
Sex				
Sex available	44,567	97.7	73,366,496	99.3
Sex NR[a]	1,058	2.3	517,312	*< 1*
Sex missing[b]	*0*	*0*	1,338	*< 1*
Race and ethnicity				
Race or ethnicity available	27,794	60.9	51,597,935	69.8
Race or ethnicity NR[a]	13,018	28.5	17,482,763	23.7
Race or ethnicity missing[c]	4,813	10.5	4,804,448	6.5

Notes. [a] We did not report sex and race/ethnicity data for Mississippi, which had unusable data quality in terms of linking of claims to Medicaid members in the demographic data file. We also did not report race/ethnicity data from states that have unusable or high concern data quality for race/ethnicity information, including Arizona, Connecticut, District of Columbia, Iowa, Louisiana, Massachusetts, New York, Oregon, Rhode Island, South Carolina, Tennessee, and Wyoming.

[b] Missing in states for which sex data is reported. [c] Missing in states for which race/ethnicity data is reported.

Abbreviations. NR: not reported; SCD: sickle cell disease.

EXHIBIT C2

Demographic characteristics: Medicaid members aged 4 to 64 with and without sickle cell disease, by age, 2021

	Members with SCD								Members without SCD			
	Aged 4 to 11		Aged 12 to 17		Aged 18 to 34		Aged 35 to 64		Aged 4 to 11	Aged 12 to 17	Aged 18 to 34	Aged 35 to 64
State	n	%	n	%	n	%	n	%	%	%	%	%
United States	*11,249*	*25*	*8,177*	*18*	*16,750*	*37*	*8,863*	*19*	*22*	*16*	--	--
Alabama[a]	--	--	--	--	--	--	--	--	--	--	--	--
Alaska[b]	--	--	--	--	--	--	--	--	--	--	--	--
Arizona	92	22	69	17	156	38	98	24	20	16	31	33
Arkansas	174	31	126	22	182	32	87	15	23	17	30	30
California	469	18	330	13	1,042	40	765	29	17	14	34	35
Colorado	55	25	36	16	85	39	44	20	20	15	33	32
Connecticut	125	21	121	20	231	39	121	20	17	13	32	37
Delaware	85	32	55	21	90	34	38	14	21	15	31	33
District of Columbia	82	22	66	17	148	39	83	22	18	11	33	38
Florida	1,271	30	861	21	1,433	34	629	15	31	22	26	20
Georgia	942	30	756	24	1,062	33	422	13	34	25	26	15
Hawaii[b]	--	--	--	--	--	--	--	--	--	--	--	--
Idaho[b]	--	--	--	--	--	--	--	--	--	--	--	--
Illinois	472	22	328	16	835	40	472	22	21	16	32	31
Indiana	199	27	123	17	300	41	116	16	22	16	31	31
Iowa	71	32	31	14	87	39	36	16	24	17	31	28
Kansas	38	26	33	22	57	39	19	13	37	26	24	13
Kentucky	107	28	64	17	135	36	74	19	19	14	31	37
Louisiana	512	26	352	18	764	38	358	18	21	16	32	31
Maine[b]	--	--	--	--	--	--	--	--	--	--	--	--
Maryland	504	27	340	18	684	37	317	17	21	16	32	31
Massachusetts	249	27	190	20	319	34	172	18	18	14	32	36
Michigan	328	21	227	15	629	41	362	23	19	14	33	34
Minnesota	124	29	84	19	147	34	76	18	23	16	31	29

	Members with SCD								Members without SCD			
	Aged 4 to 11		Aged 12 to 17		Aged 18 to 34		Aged 35 to 64		Aged 4 to 11	Aged 12 to 17	Aged 18 to 34	Aged 35 to 64
State	n	%	n	%	n	%	n	%	%	%	%	%
Mississippi	331	31	255	24	323	31	149	14	37	28	21	14
Missouri	188	27	157	22	235	34	118	17	30	21	26	22
Montana[b]	--	--	--	--	--	--	--	--	--	--	--	--
Nebraska	--	--	--	--	45	41	--	--	30	21	27	22
Nevada	81	19	48	11	171	41	118	28	22	16	30	32
New Hampshire[b]	--	--	--	--	--	--	--	--	--	--	--	--
New Jersey	325	23	259	19	523	38	281	20	22	16	31	31
New Mexico[b]	--	--	--	--	--	--	--	--	--	--	--	--
New York	936	20	660	14	1,772	38	1,245	27	16	12	33	39
North Carolina	552	28	429	21	713	36	308	15	27	20	31	23
North Dakota[b]	--	--	--	--	--	--	--	--	--	--	--	--
Ohio	423	23	301	16	721	39	386	21	21	15	31	33
Oklahoma	70	24	55	19	123	42	42	14	28	20	28	23
Oregon	13	14	20	21	40	43	21	22	18	13	33	36
Pennsylvania	496	22	396	18	869	39	458	21	21	16	31	32
Rhode Island	37	25	26	18	60	41	25	17	19	14	32	35
South Carolina	424	28	324	22	521	35	224	15	27	20	30	24
South Dakota[b]	--	--	--	--	--	--	--	--	--	--	--	--
Tennessee	277	26	218	21	372	35	195	18	29	21	29	21
Texas	909	30	663	22	975	32	504	17	37	26	26	11
Utah[a]	--	--	--	--	--	--	--	--	--	--	--	--
Vermont[b]	--	--	--	--	--	--	--	--	--	--	--	--
Virginia	302	23	237	18	503	39	249	19	22	16	31	31
Washington	73	25	41	14	114	40	59	21	21	16	32	31
West Virginia[b]	--	--	--	--	--	--	--	--	--	--	--	--
Wisconsin	146	25	97	17	198	35	132	23	22	16	32	29
Wyoming[b]	--	--	--	--	--	--	--	--	--	--	--	--

Notes. [a] Data not available. [b] Data suppressed (subgroup size < 11 or total N < 50).

EXHIBIT C3

Demographic characteristics: Medicaid members aged 4 to 64 with and without sickle cell disease, by sex, 2021

	Members with SCD				Members without SCD	
	Female		Male		Female	Male
State	n	%	n	%	%	%
United States	*24,548*	*55*	*20,019*	*45*	*55*	*45*
Alabama[a]	--		--		--	--
Alaska[b]	--		--		--	--
Arizona	229	55	186	45	53	47
Arkansas	305	54	264	46	55	45
California	1,422	55	1,184	45	54	46
Colorado	117	53	103	47	52	48
Connecticut	332	56	266	44	53	47
Delaware	157	59	111	41	54	46
District of Columbia	210	55	169	45	52	48
Florida	2,324	55	1,870	45	56	44
Georgia	1,746	55	1,436	45	59	41
Hawaii[b]	--		--		--	--
Idaho[b]	--		--		--	--
Illinois	1,168	55	939	45	54	46
Indiana	396	54	342	46	55	45
Iowa	115	51	110	49	54	46
Kansas	91	62	56	38	56	44
Kentucky	222	58	158	42	53	47
Louisiana	1,100	55	886	45	56	44
Maine[b]	--		--		--	--
Maryland	1,015	55	830	45	54	46
Massachusetts	478	51	452	49	53	47
Michigan	849	55	697	45	53	47
Minnesota	225	52	206	48	54	46
Mississippi[a]	--		--		--	--
Missouri	373	53	325	47	58	42
Montana[b]	--		--		--	--
Nebraska	53	49	56	51	56	44
Nevada	256	61	162	39	54	46
New Hampshire[b]	--		--		--	--
New Jersey	757	55	631	45	54	46
New Mexico[b]	--		--		--	--
New York	2,488	54	2,125	46	52	48

	Members with SCD				Members without SCD	
	Female		Male		Female	Male
State	n	%	n	%	%	%
North Carolina	1,131	56	871	44	58	42
North Dakota[b]	--		--		--	--
Ohio	1,012	55	819	45	54	46
Oklahoma	170	59	120	41	56	44
Oregon	42	45	52	55	53	47
Pennsylvania	1,206	54	1,013	46	53	47
Rhode Island	75	51	73	49	53	47
South Carolina	837	56	656	44	59	41
South Dakota[b]	--		--		--	--
Tennessee	630	59	432	41	59	41
Texas	1,717	56	1,334	44	59	41
Utah[a]	--		--		--	--
Vermont[b]	--		--		--	--
Virginia	700	54	591	46	55	45
Washington	157	55	130	45	53	47
West Virginia[b]	--		--		--	--
Wisconsin	313	55	260	45	54	46
Wyoming[b]	--		--		--	--

Notes. [a] Data not available. [b] Data suppressed (subgroup size < 11 or total N < 50)

EXHIBIT C4

Demographic characteristics: Medicaid members aged 4 to 64 with and without sickle cell disease, by race and ethnicity, 2021

	Members with SCD									Members without SCD				
	Missing race and ethnicity	Black, non-Hispanic		White, non-Hispanic		Other race, non-Hispanic		Hispanic		Missing race and ethnicity	Black, non-Hispanic	White, non-Hispanic	Other race, non-Hispanic	Hispanic
State	%	n	%	n	%	n	%	n	%	%	%	%	%	%
United States	*39*	*24,865*	*89*	*857*	*3*	*497*	*2*	*1,575*	*6*	*30*	*21*	*42*	*8*	*30*
Alabama[a]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
Alaska[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
Arizona	100	--	--	--	--	--	--	--	--	100	--	--	--	--
Arkansas	7	480	91	--	--	--	--	--	--	14	25	64	3	8
California	10	1,987	84	71	3	41	2	256	11	10	9	21	11	60
Colorado	0	166	75	--	--	--	--	--	--	1	7	41	18	34
Connecticut	100	--	--	--	--	--	--	--	--	100	--	--	--	--
Delaware	0	243	91	--	--	--	--	--	--	0	38	41	3	19
District of Columbia	100	--	--	--	--	--	--	--	--	100	--	--	--	--
Florida	34	2,385	87	30	1	17	1	320	12	15	29	31	2	38
Georgia	25	2,285	95	50	2	38	2	20	1	6	50	42	5	3
Hawaii[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
Idaho[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
Illinois	3	1,937	94	69	3	12	1	33	2	5	31	48	5	15
Indiana	35	473	98	--	--	--	--	--	--	18	22	71	0	7
Iowa	100	--	--	--	--	--	--	--	--	100	--	--	--	--
Kansas	0	117	80	--	--	--	--	--	--	5	10	53	10	27
Kentucky	17	288	92	--	--	--	--	--	--	15	13	79	3	4
Louisiana	100	--	--	--	--	--	--	--	--	100	--	--	--	--
Maine[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
Maryland	17	1,410	92	22	1	49	3	51	3	18	45	29	8	19

	Members with SCD									Members without SCD				
	Missing race and ethnicity	Black, non-Hispanic		White, non-Hispanic		Other race, non-Hispanic		Hispanic		Missing race and ethnicity	Black, non-Hispanic	White, non-Hispanic	Other race, non-Hispanic	Hispanic
State	%	n	%	n	%	n	%	n	%	%	%	%	%	%
Massachusetts	100	--	--	--	--	--	--	--	--	100	--	--	--	--
Michigan	3	1,386	92	54	4	52	3	13	1	4	28	59	6	7
Minnesota	7	380	95	--	--	--	--	--	--	9	24	53	13	10
Mississippi[a]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
Missouri	5	610	92	--	--	--	--	--	--	6	23	64	2	11
Montana[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
Nebraska	0	86	79	--	--	--	--	--	--	6	14	54	7	24
Nevada	3	361	89	--	--	--	--	--	--	3	21	33	8	38
New Hampshire[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
New Jersey	6	1,071	82	79	6	12	1	142	11	8	26	39	6	30
New Mexico[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
New York	100	--	--	--	--	--	--	--	--	100	--	--	--	--
North Carolina	1	1,829	92	38	2	64	3	60	3	0	35	43	7	15
North Dakota[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
Ohio	3	1,691	95	44	2	16	1	31	2	6	30	61	3	5
Oklahoma	6	244	89	--	--	--	--	--	--	7	11	49	23	17
Oregon	100	--	--	--	--	--	--	--	--	100	--	--	--	--
Pennsylvania	4	1,787	84	88	4	24	1	221	10	6	26	55	4	15
Rhode Island	100	--	--	--	--	--	--	--	--	100	--	--	--	--
South Carolina	100	--	--	--	--	--	--	--	--	100	--	--	--	--
South Dakota[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
Tennessee	100	--	--	--	--	--	--	--	--	100	--	--	--	--
Texas	35	1,681	84	63	3	23	1	228	11	11	17	19	3	60
Utah[a]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
Vermont[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--

	Members with SCD									Members without SCD				
	Missing race and ethnicity		Black, non-Hispanic		White, non-Hispanic		Other race, non-Hispanic		Hispanic	Missing race and ethnicity	Black, non-Hispanic	White, non-Hispanic	Other race, non-Hispanic	Hispanic
State	%	n	%	n	%	n	%	n	%	%	%	%	%	%
Virginia	2	1,162	92	59	5	24	2	20	2	6	37	54	6	4
Washington	6	219	81	--	--	--	--	--	--	6	8	54	14	24
West Virginia[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--
Wisconsin	15	460	95	--	--	--	--	--	--	8	20	56	10	14
Wyoming[b]	--	--	--	--	--	--	--	--	--	--	--	--	--	--

Notes. [a] Data not available. [b] Data suppressed (subgroup size < 11 or total N < 50).

APPENDIX D
HEALTH CARE SERVICE USE

EXHIBIT D1
Prevalence of affected body systems and specific conditions in Medicaid members aged 4 to 64 years, 2021

System or condition	Medicaid members with SCD	%[a]	Matched Medicaid members without SCD[b]	%[a]
Total members[a]	*45,620*	-	*136,860*	-
Hematological	45,620	100.0	1,400	1.0
Pulmonary	15,380	33.7	11,195	8.2
Cardiovascular	14,815	32.5	12,077	8.8
Skeletal	11,113	24.4	8,805	6.4
Psychological	9,275	20.3	19,543	14.2
Gastroenterological	6,282	13.8	6,694	4.9
Renal	3,907	8.6	2,844	2.1
Stroke[c]	1,944	4.3	641	< 1

[a] No health care information available in 2021 for 5 members identified as having SCD. For the purposes of this calculation, they were eliminated from analysis along with their matched counterparts. [b] Medicaid members without SCD matched to members with SCD at 3:1 on state, age, sex, race, and ethnicity. [c] Stroke or transient ischemic attack.

EXHIBIT D2
Health service use by Medicaid members aged 4 to 64 years, 2021

	Medicaid members with SCD	Matched Medicaid members without SCD
Total members	*45,625*	*136,875*
Hospitalizations		
% with ≥ 1 hospitalization	44.1	6.0
% with ≥ 2 hospitalizations	23.0	1.3
Total hospitalizations, per 1,000 members	1,287	86
Total inpatient days, per 1,000 members	7,056	445
Average length of stay per hospitalization, days	5.5	5.2
% with ≥ 1 hospitalization lasting ≥ 5 days	22.5	1.9
Emergency visits		
% with ≥ 1 ED visit	66.3	27.2
% with ≥ 5 ED visits	18.4	2.0
Total ED visits, per 1,000 members	3,565	556

Note. [a] Medicaid members without SCD matched to members with SCD at 3:1 on state, age, sex, race, and ethnicity.
Abbreviations. ED: emergency department; SCD: sickle cell disease.

EXHIBIT D3

Health care service use: hospitalization, 2021

State	Members with ≥ 1 hospitalizations, %		Members with ≥ 2 hospitalizations, %		Hospitalizations per 1,000 members	
	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD
United States	*44*	*6*	*23*	*1*	*1,287*	*86*
Alabama[a]	--	--	--	--	--	--
Alaska[b]	--	--	--	--	--	--
Arizona	51	6	28	1	1,508	73
Arkansas	41	3	22	1	1,162	45
California	48	5	25	1	1,414	77
Colorado	46	3	19	--	1,027	36
Connecticut	45	6	24	1	1,226	81
Delaware	43	4	18	--	910	68
District of Columbia	45	4	26	--	1,467	54
Florida	47	7	25	2	1,420	99
Georgia	43	7	23	1	1,262	99
Hawaii[b]	--	--	--	--	--	--
Idaho[b]	--	--	--	--	--	--
Illinois	44	6	22	1	1,297	94
Indiana	44	5	31	2	1,944	76
Iowa	41	4	21	--	1,164	40
Kansas	44	10	24	3	1,517	161
Kentucky	44	6	21	--	1,166	78
Louisiana	41	5	21	1	1,240	68
Maine[b]	--	--	--	--	--	--
Maryland	37	5	18	1	904	64
Massachusetts	39	4	20	1	1,129	91
Michigan	45	6	22	1	1,215	78
Minnesota	47	6	22	1	1,084	77
Mississippi	37	8	19	1	1,061	101
Missouri	49	9	27	2	1,620	125
Montana[b]	--	--	--	--	--	--
Nebraska	39	8	24	--	1,514	104
Nevada	52	8	30	2	1,904	136
New Hampshire[b]	--	--	--	--	--	--
New Jersey	44	5	23	1	1,356	76
New Mexico[b]	--	--	--	--	--	--
New York	47	6	25	2	1,496	90

State	Members with ≥ 1 hospitalizations, %		Members with ≥ 2 hospitalizations, %		Hospitalizations per 1,000 members	
	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD
North Carolina	43	5	22	1	1,241	65
North Dakota[b]	--	--	--	--	--	--
Ohio	48	7	26	2	1,531	101
Oklahoma	57	7	35	2	1,679	91
Oregon	40	--	19	--	1,287	60
Pennsylvania	45	6	23	1	1,277	88
Rhode Island	16	--	7	--	284	25
South Carolina	38	4	20	1	1,073	55
South Dakota[b]	--	--	--	--	--	--
Tennessee	41	8	22	2	1,090	107
Texas	44	7	22	1	1,056	97
Utah[a]	--	--	--	--	--	--
Vermont[b]	--	--	--	--	--	--
Virginia	39	6	20	1	1,093	81
Washington	45	5	20	--	1,146	60
West Virginia[b]	--	--	--	--	--	148
Wisconsin	43	6	21	1	1,127	96
Wyoming[b]	--	--	--	--	--	--

Notes. [a] Data not available. [b] Data suppressed (subgroup size < 11 or total N < 50).

EXHIBIT D4

Health care service use: intensity of hospital use, 2021

State	Inpatient days per 1,000 members		Average length of stay per hospitalization, days		Share of hospitalized members whose stay lasted ≥ 5 days, %	
	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD
United States	*7,056*	*445*	*5.5*	*5.2*	*23*	*2*
Alabama[a]	--	--	--	--	--	--
Alaska[b]	--	--	--	--	--	--
Arizona	9,634	357	6.4	4.9	27	2
Arkansas	5,873	237	5.1	5.3	20	1
California	7,270	355	5.1	4.6	22	1
Colorado	6,686	176	6.5	4.8	20	--
Connecticut	6,048	362	4.9	4.5	21	2
Delaware	7,075	359	7.8	5.3	17	2
District of Columbia	8,016	216	5.5	4.0	26	1
Florida	7,062	504	5.0	5.1	24	2
Georgia	6,473	509	5.1	5.2	21	2
Hawaii[b]	--	--	--	--	--	--
Idaho[b]	--	435	--	0.0	--	--
Illinois	8,088	464	6.2	5.0	24	2
Indiana	11,069	360	5.7	4.7	34	2
Iowa	7,996	124	6.9	3.1	24	--
Kansas	9,673	900	6.4	5.6	26	3
Kentucky	4,463	186	3.8	2.4	17	--
Louisiana	6,223	294	5.0	4.3	21	2
Maine[b]	--	--	--	--	--	--
Maryland	5,251	299	5.8	4.7	18	2
Massachusetts	7,084	451	6.3	4.9	22	2
Michigan	6,972	375	5.7	4.8	22	2
Minnesota	5,777	544	5.3	7.1	21	2
Mississippi	4,934	443	4.6	4.4	18	2
Missouri	7,731	684	4.8	5.5	23	3
Montana[b]	--	--	--	--	--	--
Nebraska	8,000	691	5.3	6.6	22	--
Nevada	8,976	742	4.7	5.5	25	2
New Hampshire[b]	--	362	--	0.0	--	--
New Jersey	7,839	568	5.8	7.4	25	2
New Mexico[b]	--	342	--	0.0	--	--

State	Inpatient days per 1,000 members		Average length of stay per hospitalization, days		Share of hospitalized members whose stay lasted ≥ 5 days, %	
	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD
New York	8,651	471	5.8	5.2	25	2
North Carolina	6,112	331	4.9	5.1	21	1
North Dakota[b]	--	--	--	--	--	--
Ohio	8,581	478	5.6	4.7	24	2
Oklahoma	8,979	477	5.3	5.3	27	1
Oregon	11,106	337	8.6	5.6	19	--
Pennsylvania	7,534	551	5.9	6.3	22	2
Rhode Island	2,101	86	7.4	3.5	--	--
South Carolina	5,939	328	5.5	6.0	21	1
South Dakota[b]	--	--	--	--	--	--
Tennessee	6,627	616	6.1	5.8	22	2
Texas	5,645	568	5.3	5.8	22	2
Utah[a]	--	--	--	--	--	--
Vermont[b]	--	--	--	--	--	--
Virginia	6,529	396	6.0	4.9	23	2
Washington	7,059	187	6.2	3.1	20	1
West Virginia[b]	--	2,207	--	14.9	--	--
Wisconsin	5,052	480	4.5	5.0	19	2
Wyoming[b]	--	--	--	--	--	--

Notes. [a] Data not available. [b] Data suppressed (subgroup size < 11 or total N < 50).

EXHIBIT D5

Health care service use: emergency department use, 2021

State	Members with ≥ 1 ED visits, %		Members with ≥ 5 ED visits, %		ED visits per 1,000 members	
	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD
United States	*66*	*27*	*18*	*2*	*3,565*	*556*
Alabama[a]	--	--	--	--	--	--
Alaska[b]	--	--	--	--	--	--
Arizona	70	20	18	1	3,092	333
Arkansas	66	20	18	1	3,118	359
California	66	25	19	2	3,369	528
Colorado	64	27	16	3	2,632	580
Connecticut	65	29	17	2	3,206	555
Delaware	68	25	13	2	2,530	515
District of Columbia	72	27	22	2	4,216	585
Florida	71	30	20	2	3,988	628
Georgia	70	25	20	2	3,853	546
Hawaii[b]	--	--	--	--	--	--
Idaho[b]	--	--	--	--	--	319
Illinois	33	13	4	--	937	178
Indiana	70	32	21	2	3,986	645
Iowa	62	23	20	--	3,400	431
Kansas	65	37	22	4	5,306	862
Kentucky	69	30	15	2	3,221	637
Louisiana	74	33	24	2	4,745	656
Maine[b]	--	--	--	--	--	281
Maryland	61	24	13	2	2,359	464
Massachusetts	62	23	13	1	2,686	429
Michigan	64	31	18	3	3,779	663
Minnesota	71	26	18	2	3,244	522
Mississippi	68	32	21	2	4,212	662
Missouri	68	34	21	3	3,596	733
Montana[b]	--	--	--	--	--	--
Nebraska	64	32	17	--	5,266	590
Nevada	77	35	32	3	5,347	796
New Hampshire[b]	--	--	--	--	--	594
New Jersey	70	32	18	2	3,502	656
New Mexico[b]	--	--	--	--	--	608
New York	65	22	16	1	3,530	423
North Carolina	67	24	20	2	3,532	469

State	Members with ≥ 1 ED visits, %		Members with ≥ 5 ED visits, %		ED visits per 1,000 members	
	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD	Members aged 4 to 64 with SCD	Matched members without SCD
North Dakota[b]	--	--	--	--	--	--
Ohio	73	41	24	4	4,392	925
Oklahoma	72	30	25	2	6,217	589
Oregon	66	18	17	--	2,862	401
Pennsylvania	69	30	18	2	3,511	610
Rhode Island	30	14	--	--	1,743	273
South Carolina	67	21	19	1	4,195	422
South Dakota[b]	--	--	--	--	--	--
Tennessee	64	29	17	2	3,292	566
Texas	69	27	20	3	3,484	600
Utah[a]	--	--	--	--	--	--
Vermont[b]	--	--	--	--	--	--
Virginia	68	32	20	2	3,755	660
Washington	69	27	17	2	3,216	530
West Virginia[b]	--	--	--	--	--	778
Wisconsin	73	36	24	3	4,749	785
Wyoming[b]	--	--	--	--	--	--

Notes. [a] Data not available. [b] Data suppressed (subgroup size < 11 or total N < 50).

APPENDIX E
VOXELOTOR UPTAKE

EXHIBIT E1

Drug uptake

State	Members with SCD, aged 12 and older, n	Members with voxelotor claim, n	Drug uptake, %
United States	--	--	--
Alabama[a]	--	--	--
Alaska[b]	--	--	--
Arizona	323	50	15.5
Arkansas	395	11	2.8
California	2,138	147	6.9
Colorado[b]	165	--	--
Connecticut	473	43	9.1
Delaware[b]	183	--	--
District of Columbia	297	18	6.1
Florida	2,923	226	7.7
Georgia	2,240	113	5.0
Hawaii[b]	--	--	--
Idaho[b]	--	--	--
Illinois	1,635	60	3.7
Indiana	539	27	5.0
Iowa[b]	154	--	--
Kansas[b]	109	--	--
Kentucky	273	16	5.9
Louisiana	1,474	150	10.2
Maine[b]	--	--	--
Maryland	1,341	74	5.5
Massachusetts	681	32	4.7
Michigan	1,218	59	4.8
Minnesota	307	20	6.5
Mississippi	727	31	4.3
Missouri	510	29	5.7
Montana[b]	--	--	--
Nebraska	73	--	--
Nevada	337	21	6.2
New Hampshire[b]	--	--	--
New Jersey	1,063	102	9.6
New Mexico[b]	--	--	--
New York	3,677	256	7.0

State	Members with SCD, aged 12 and older, n	Members with voxelotor claim, n	Drug uptake, %
North Carolina	1,450	95	6.6
North Dakota[b]	--	--	--
Ohio	1,408	92	6.5
Oklahoma	220	16	7.3
Oregon	81	--	--
Pennsylvania	1,723	77	4.5
Rhode Island	111	23	20.7
South Carolina	1,069	124	11.6
South Dakota[b]	--	--	--
Tennessee	785	45	5.7
Texas	2,142	143	6.7
Utah[a]	--	--	--
Vermont[b]	--	--	--
Virginia	989	103	10.4
Washington	214	17	7.9
West Virginia[b]	--	--	--
Wisconsin	427	12	2.8
Wyoming[b]	--	--	--

Notes. [a] Data not available. [b] Data suppressed (subgroup size < 11 or total N < 50).

EXHIBIT E2

Medication adherence, means, and proportions, stratified by enrollment status, 2021

	Total members	Mean MPR	Adherent[c],%	Not adherent, %
Fully enrolled[a]	1,629	0.62	35.8	64.2
Not fully enrolled	79	0.47	22.8	77.2
Missing enrollment information[b]	117	0.56	26.5	73.5
All members with voxelotor fills	1,825	0.61	34.6	65.3

Notes. [a] Fully enrolled members are those with calculated 365 days of enrollment in the year 2021, not fully enrolled members have less than 365 days of calculated enrollment. [b] Enrollment information was missing for 117 members with claims that could not be linked to the Demographic Eligibility data file. See Data Methods for detail. [c] Greater than or equal to 0.8 MPR.
Abbreviations. MPR: medication possession ratio.

APPENDIX F

FORECASTED ANNUAL COST OF VOXELOTOR BY STATE

EXHIBIT F

Forecasted annual cost of voxelotor (in millions) by state

State	Total			Per member per month[c], $	State contribution[d], $	Federal contribution[d], $
	Forecasted spending, $	95% estimate bounds				
		Lower, $	Upper, $			
United States	*388.1*	*197.5*	*794.2*	*0.4*	*129.5*	*258.6*
Alabama[a]	12.1	6.2	24.6	1.0	3.2	8.9
Alaska[b]	--	--	--	--	--	--
Arizona	3.4	1.7	7.1	0.1	0.9	2.5
Arkansas	4.7	2.4	9.6	0.4	1.2	3.4
California	21.5	10.8	43.6	0.1	8.4	13.1
Colorado	1.8	0.9	3.7	0.1	0.7	1.1
Connecticut	4.9	2.5	10.0	0.5	1.8	3.1
Delaware	2.2	1.1	4.6	0.7	0.8	1.4
District of Columbia	3.1	1.6	6.4	1.1	0.8	2.3
Florida	34.5	17.5	69.8	0.8	14.5	20.1
Georgia	26.2	12.9	52.9	1.0	8.7	17.5
Hawaii[b]	--	--	--	--	--	--
Idaho[b]	--	--	--	--	--	--
Illinois	17.3	8.7	35.2	0.5	7.0	10.4
Indiana	6.1	3.1	12.0	0.3	1.9	4.2
Iowa	1.9	0.9	3.7	0.2	0.6	1.3
Kansas	1.2	0.6	2.4	0.3	0.5	0.7
Kentucky	--	--	--	--	--	--
Louisiana	16.4	8.5	33.4	0.8	4.4	11.9
Maine[b]	--	--	--	--	--	--
Maryland	15.2	7.7	31.1	0.9	6.1	9.0
Massachusetts	7.7	3.7	15.4	0.4	3.3	4.4
Michigan	12.7	6.5	26.6	0.4	3.8	8.9
Minnesota	3.5	1.8	7.2	0.3	1.5	2.1
Mississippi	8.7	4.4	17.6	1.1	1.9	6.8
Missouri	5.7	2.9	11.6	0.5	1.9	3.8
Montana[b]	--	--	--	--	--	--
Nebraska	0.9	0.5	1.8	0.3	0.3	0.6
Nevada	3.4	1.8	7.0	0.4	1.0	2.4
New Hampshire[b]	--	--	--	--	--	--
New Jersey	11.4	5.8	23.5	0.5	4.4	7.1

State	Total					
	Forecasted spending, $	95% estimate bounds		Per member per month[c], $	State contribution[d], $	Federal contribution[d], $
		Lower, $	Upper, $			
New Mexico[b]	--	--	--	--	--	--
New York	38.0	19.1	78.0	0.5	14.5	23.4
North Carolina	16.5	8.3	33.3	0.6	5.5	11.0
North Dakota[b]	--	--	--	--	--	--
Ohio	15.1	7.7	31.0	0.5	4.6	10.4
Oklahoma	2.4	1.2	4.9	0.2	0.7	1.7
Oregon	0.8	0.4	1.6	0.1	0.2	0.6
Pennsylvania	18.3	9.4	36.8	0.5	7.1	11.2
Rhode Island	1.2	0.6	2.5	0.3	0.4	0.8
South Carolina	12.3	6.3	24.6	0.8	3.7	8.6
South Dakota[b]	--	--	--	--	--	--
Tennessee	8.7	4.4	17.7	0.5	3.0	5.7
Texas	25.1	12.8	51.0	0.4	9.8	15.3
Utah[b]	--	--	--	--	--	--
Vermont[b]	--	--	--	--	--	--
Virginia	10.6	5.3	21.3	0.5	4.3	6.4
Washington	2.4	1.2	4.9	0.1	1.0	1.4
West Virginia[b]	--	--	--	--	--	--
Wisconsin	4.7	2.4	9.6	0.3	1.8	2.9
Wyoming[b]	--	--	--	--	--	--

Notes. [a] Cost estimates for Alabama are based on prevalence estimates obtained from.[14] See Methods Appendix for more detail. [b] Not calculated. (N < 50). [c] Excluding dual enrollment. Member-month counts for Alabama, Mississippi, and Tennessee are obtained from another source [17] due to data availability or reliability issues in TAF. [d] Based on the share of the members with SCD drug indication in Medicaid, Medicaid Expansion, and CHIP enrollment categories in each state in 2021. For states with unusable data quality for identifying expansion enrollment (i.e., Idaho, Illinois, and Virginia), the average expansion enrollment percentages in other expansion states are used. For states with unusable data quality for identifying CHIP enrollment (i.e., Arkansas, Kentucky, and North Dakota), the average CHIP enrollment percentages in all states are used.

REFERENCES

1. Medicaid.gov. Medicaid data quality (DQ) atlas. 2024; https://www.medicaid.gov/dq-atlas/welcome. Accessed April 12, 2024.

2. Centers for Medicare and Medicaid Services. TAF technical guidance: Claims files. 2022; https://resdac.org/sites/datadocumentation.resdac.org/files/2022-06/TAF-TechGuide-Claims-Files.pdf. Accessed April 12, 2024.

3. Reeves S, Garcia E, Kleyn M, et al. Identifying sickle cell disease cases using administrative claims. *Academic pediatrics.* 2014;14(5 Suppl):S61-67.

4. Kang HA, Barner JC, Richards KM, Bhor M, Paulose J, Kutlar A. Association between vaso-occlusive crises and opioid prescriptions among patients with sickle cell disease: A retrospective claims-based study. *Journal of Health Economics & Outcomes Research.* 2020;7(1):94-101.

5. Brousseau DC, Richardson T, Hall M, et al. Hydroxyurea use for sickle cell disease among Medicaid-enrolled children. *Pediatrics.* 2019;144(1):07.

6. Shankar SM, Arbogast PG, Mitchel E, Cooper WO, Wang WC, Griffin MR. Medical care utilization and mortality in sickle cell disease: a population-based study. *American Journal of Hematology.* 2005;80(4):262-270.

7. Mvundura M, Amendah D, Kavanagh PL, Sprinz PG, Grosse SD. Health care utilization and expenditures for privately and publicly insured children with sickle cell disease in the United States. *Pediatric Blood & Cancer.* 2009;53(4):642-646.

8. US Food & Drug Administration. National drug code directory. 2024; https://www.accessdata.fda.gov/scripts/cder/ndc/index.cfm. Accessed April 12, 2024.

9. Kronick R, Gilmer T, Dreyfus T, Lee L. Improving health-based payment for Medicaid beneficiaries: CDPS. *Health Care Financ Rev.* 2000;21(3):29-64.

10. Biospace. Oxbryta (voxelotor) tablets for oral suspension, a new dispersible tablet dosage form, now available for patients with sickle cell disease in the united states. 2022; https://www.biospace.com/article/releases/oxbryta-voxelotor-tablets-for-oral-suspension-a-new-dispersible-tablet-dosage-form-now-available-for-patients-with-sickle-cell-disease-in-the-united-states-/. Accessed April 12, 2024.

11. Newman TV, Yang J, Suh K, Jonassaint CR, Kane-Gill SL, Novelli EM. Use of disease-modifying treatments in patients with sickle cell disease. *JAMA Netw Open.* 2023;6(11):e2344546. doi: 10.1001/jamanetworkopen.2023.44546.

12. US Social Security Administration. Payment for covered outpatient drugs. 1990; https://www.ssa.gov/OP_Home/ssact/title19/1927.htm. Accessed May 15, 2024.

13. Vichinsky E, Hoppe CC, Ataga KI, et al. A phase 3 randomized trial of voxelotor in sickle cell disease. *N Engl J Med.* 2019;381(6):509-519. doi: 10.1056/NEJMoa1903212.

14. NORC at the University of Chicago. New analysis of sickle cell disease prevalence among Medicaid enrollees fills gap in public data. 2023; https://www.norc.org/research/library/spotlight-new-analysis-of-sickle-cell-disease-prevalence-among-medicaid-enrollees.html. Accessed December 7, 2023.

15. Medicaid and CHIP payment and access commission. MACStats: Medicaid and CHIP data book. 2023; https://www.macpac.gov/wp-content/uploads/2023/12/MACSTATS_Dec2023_WEB-508.pdf. Accessed April 12, 2024.

16. Medicaid and CHIP payment and access commission. Federal match rate exceptions. 2024; https://www.macpac.gov/federal-match-rate-exceptions/. Accessed April 12, 2024.

17. KFF. Medicaid and CHIP monthly enrollment. 2022; https://www.kff.org/other/state-indicator/medicaid-and-chip-monthly-enrollment/?currentTimeframe=23&selectedRows=%7B%22states%22:%7B%22alabama%22:%7B%7D%7D%7D&sortModel=%7B%22colId%22:%22Location%22,%22sort%22:%22asc%22%7D. Accessed April 12, 2024.

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