High-Expenditure Pharmaceutical Use Among Children in Medicaid

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abstract

BACKGROUND AND OBJECTIVES: Medication use may be a target for quality improvement, cost containment, and research. We aimed to identify medication classes associated with the highest expenditures among pediatric Medicaid enrollees and to characterize the demographic, clinical, and health service use of children prescribed these medications.

METHODS: Retrospective, cross-sectional study of 3 271 081 Medicaid-enrolled children. Outpatient medication spending among high-expenditure medication classes, defined as the 10 most expensive among 261 mutually exclusive medication classes, was determined by using transaction prices paid to pharmacies by Medicaid agencies and managed care plans among prescriptions filled and dispensed in 2013.

RESULTS: Outpatient medications accounted for 16.6% of all Medicaid expenditures. The 10 most expensive medication classes accounted for 63.9% of all medication expenditures. Stimulants (amphetamine-type) accounted for both the highest proportion of expenditures (20.6%) and days of medication use (14.0%) among medication classes. Users of medications in the 10 highest-expenditure classes were more likely to have a chronic condition of any complexity (77.9% vs 41.6%), a mental health condition (35.7% vs 11.9%), or a complex chronic condition (9.8% vs 4.3%) than other Medicaid enrollees (all $P <$ .001). The 4 medications with the highest spending were all psychotropic medications. Polypharmacy was common across all high-expenditure classes.

CONCLUSIONS: Medicaid expenditure on pediatric medicines is concentrated among a relatively small number of medication classes most commonly used in children with chronic conditions. Interventions to improve medication safety and effectiveness and contain costs may benefit from better delineation of the appropriate prescription of these medications.

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WHAT'S KNOWN ON THIS SUBJECT: Much of the analysis of health care use and spending among high-cost pediatric populations has focused on preventable hospital care. Little is known about potential contributors to pharmaceutical expenditure in pediatric populations.

WHAT THIS STUDY ADDS: Among 3.2 million Medicaid enrollees in 2013, 10 medication classes accounted for 64% of medication expenditures. Use was concentrated among individuals with chronic conditions. The 4 medications with the highest spending were psychotropic medications.

To cite: Cohen E, Hall M, Lopert R, et al. High-Expenditure Pharmaceutical Use Among Children in Medicaid. *Pediatrics.* 2017;140(3):e20171095

Medication use and spending is a central issue for patients, providers, payers, and policy makers. Prescription medications accounted for $457 billion in expenditures in the United States in 2015, or almost 17% of total health care spending.[1] Double-digit increases in expenditures have been reported over the last several years,[2,3] outpacing that of aggregate health care expenditures. Not surprisingly, this is of increasing concern to governments and other third-party payers seeking to provide high-quality health care within sustainable and, at times, fixed health care budgets.[4]

Medications are also an important contributor to overall expenditures for the >30 million pediatric Medicaid enrollees,[5] among them being a disproportionate number of pediatric Medicaid users who have chronic conditions. Of the top 1% of the most expensive children in Medicaid, pharmacy costs are nearly $700 per member per month, making this the third most-expensive category of service use (after inpatient admissions and outpatient mental health).[6] Among persistently high-cost Medicaid enrollees in California Children's Services (the largest Title V program in the United States), drug expenditures were the largest single category of service use (44% of total health care spending).[7]

For adults, concern about rising drug spending has been raised, particularly regarding expensive, new drugs for the treatment of conditions such as hepatitis C and cancer.[8] However, less is known regarding potential contributors to pharmaceutical expenditure in pediatric populations. To date, much of the analysis of health care use and spending among high-cost pediatric populations has focused on preventable hospital care.[9,10] It is thought that many hospitalizations might be preventable with better care coordination and management in outpatient and community settings. However, given the substantial contribution of medications to overall expenditures, a better understanding of medication spending may provide another target for comparative effectiveness research, quality improvement, and appropriate cost-containment strategies. In this study, we aimed to (1) identify the medication classes and specific medications associated with high expenditures among a cohort of children in Medicaid and (2) describe the demographic, clinical, and overall health service use of the children for whom the medications were prescribed.

METHODS

Study Design, Population, and Setting

This was a cross-sectional, retrospective study of the Medicaid MarketScan Database (Truven Health Analytics, Ann Arbor, MI), which contains health care claims data on children ages 0 to 18 years enrolled continuously (≥11 months) during 2013 in Medicaid fee-for-service, managed care, or the Children's Health Insurance Program from 10 deidentified states in all geographic regions of the United States. Data validity and integrity are maintained by Truven Health Analytics. This study was deemed exempt from review by the Hospital for Sick Children Research Ethics Board because we used deidentified data.

Outcome Measure

The main outcome measure was outpatient medication expenditure by medication class. Included were all noninpatient medications, including those dispensed by outpatient pharmacies as well as those dispensed directly in an office setting or the home, such as injectable medication products. Specifically, our intention was to identify high-expenditure medications, which were defined as those medication classes accounting for the largest proportions of both expenditure and use among the children in the cohort.

Because the majority (~60%) of children in the database were in Medicaid managed care,[6] direct estimates of spending on individual prescriptions were unavailable from claims data, so an indirect method of estimating the expenditures on these medications was used. We estimated the expenditure on each medication by using transaction prices paid by Medicaid agencies and managed care plans to pharmacies for prescriptions filled and dispensed to Medicaid enrollees from the national Medicaid drug use data for 2013.[11] These data include total amounts paid to pharmacies (excluding rebates), the total number of units dispensed, and the total number of prescriptions by National Drug Code (NDC) across all states, and they have been previously used in Medicaid drug expenditure research.[12–15] Expenditure was calculated by multiplying the mean per prescription payment for each NDC for each drug in this data set by the total number of prescriptions dispensed for all children in the Truven MarketScan Medicaid Database. We did not use list price benchmarks because they do not accurately represent drug costs after rebates to Medicaid and thus lead to inflated reimbursement estimates.[16] Individual medications with the highest estimated expenditures per prescription by using this method are summarized in Supplemental Table 3.

Medication Classification: Medication Groups and Classes

Medication classification was based on the NDCs reported in the 2013 version of the Red Book (Truven Health Analytics, Ann Arbor, MI), which contains detailed information on >200 000 medications. By using the NDCs, we applied the American Hospital Formulary System Pharmacologic-Therapeutic

Classification to each drug,[17] classifying medications into 1 of 31 mutually exclusive therapeutic groups, and then into 1 of 261 mutually exclusive medication classes. As an example, the drugs phenobarbital and risperidone both reside in the central nervous system therapeutic group, but phenobarbital is included in the anticonvulsant medication class whereas risperidone is a member of the antipsychotic medication class. Given that therapeutic groups could be too broad for meaningful clinical analysis (eg, central nervous system drugs could be prescribed for either a neurologic condition or a psychiatric condition), we elected to focus our analysis at the level of the medication class.

High-Expenditure Medications

We ranked the top 10 medication classes by total expenditure in 2013 as well as by total use among the cohort (defined as the total number of days of medication dispensed in 2013) informed by the Pareto principle. We also described concomitant use of other medication classes (both the number of other medication classes and the most common medication classes used). For each of the 10 medication classes with the highest expenditures, we also described the 3 individual medications (chemical moieties) with the highest expenditures. We defined "brand drugs" as those identified as single source (ie, those with no competitor on the market in 2013), whereas all multisource drugs (including branded originator drugs with generic competitors) were classified as "generics."

Demographic and Clinical Characteristics of Child Medicaid Enrollees

We described the characteristics of children using drugs in the 10 medication classes associated with the largest expenditures. Demographic characteristics included age, sex, race and/or ethnicity, and disability status as determined by Medicaid enrollment as a "blind or disabled individual." The number of comorbid chronic health conditions was ascertained by using the Agency for Healthcare Research and Quality Chronic Condition Indicator classification system,[18] which is based on *International Classification of Diseases, Ninth Revision, Clinical Modification* codes. The Chronic Condition Indicator system also creates 18 mutually exclusive clinical groupings; we calculated the total number of chronic conditions for each child and the presence or absence of a mental health chronic condition. Complex chronic conditions were defined as those lasting ≥12 months, involving either severe single-organ or multiorgan dysfunction, were probably associated with hospitalization in a tertiary care center, and were ascertained by using an established set of *International Classification of Diseases, Ninth Revision, Clinical Modification* codes.[19] For patients using each of the top 10 medication classes of interest, we ascertained their individual health care use. This use was reported as overall spending on health care by using methods previously described.[6,20] We also determined the proportion of spending attributed to index medication class as well as total pharmaceutical use, including the most common medication classes among users of polypharmacy.

Statistical Analysis

We summarized categorical variables using frequencies, percentages, and continuous variables with medians and interquartile ranges. Comparisons between enrollees using medications in the 10 medication classes associated with the largest spending and all other children who were Medicaid enrollees were conducted by using the χ^2 test for categorical data and Wilcoxon rank- tests for continuous data. All analyses were performed by using SAS version 9.4 (SAS Institute, Cary, NC).

RESULTS

A total of 3 271 081 children were included in the cohort, with 21.7 million unique prescriptions dispensed and amounting to 454.8 million days of medication use. The total estimated pharmaceutical expenditure was $1.65 billion, or 16.6% of all Medicaid expenditures.

The top 10 medication classes accounted for 63.9% of all medication expenditures and 60.8% of all days of medication dispensed (Figs 1 and 2). Of these medication classes, stimulants (amphetamine-type) accounted for both the greatest proportions of expenditures (20.6%) and use (14.0% of total days of medication dispensed). Other classes in the top 10 for expenditures included antipsychotics (9.4%), inhaled steroids (8.0%), coagulation and anticoagulation factors (4.4%), sympathomimetics (4.2%), anticonvulsants (4.2%), pituitary hormones (3.9%), antiviral agents (3.8%), α-adrenergic blockers (3.5%), and cephalosporins (1.8%). Other medication classes in the top 10 for days of use included the following: antihistamines (10.6%), sympathomimetics (5.8%), α-adrenergic blockers (5.1%), antidepressants (5.0%), inhaled steroids (4.7%), antipsychotics (4.1%), anticonvulsants (4.0%), leukotriene modifiers (3.9%), and anti-inflammatories (3.8%).

Demographic and Clinical Characteristics of Users of Each of the 10 Highest-Expenditure Medication Classes

Characteristics of the children who used the medication classes with the highest expenditure compared with all other children continuously enrolled in 2013 are summarized in

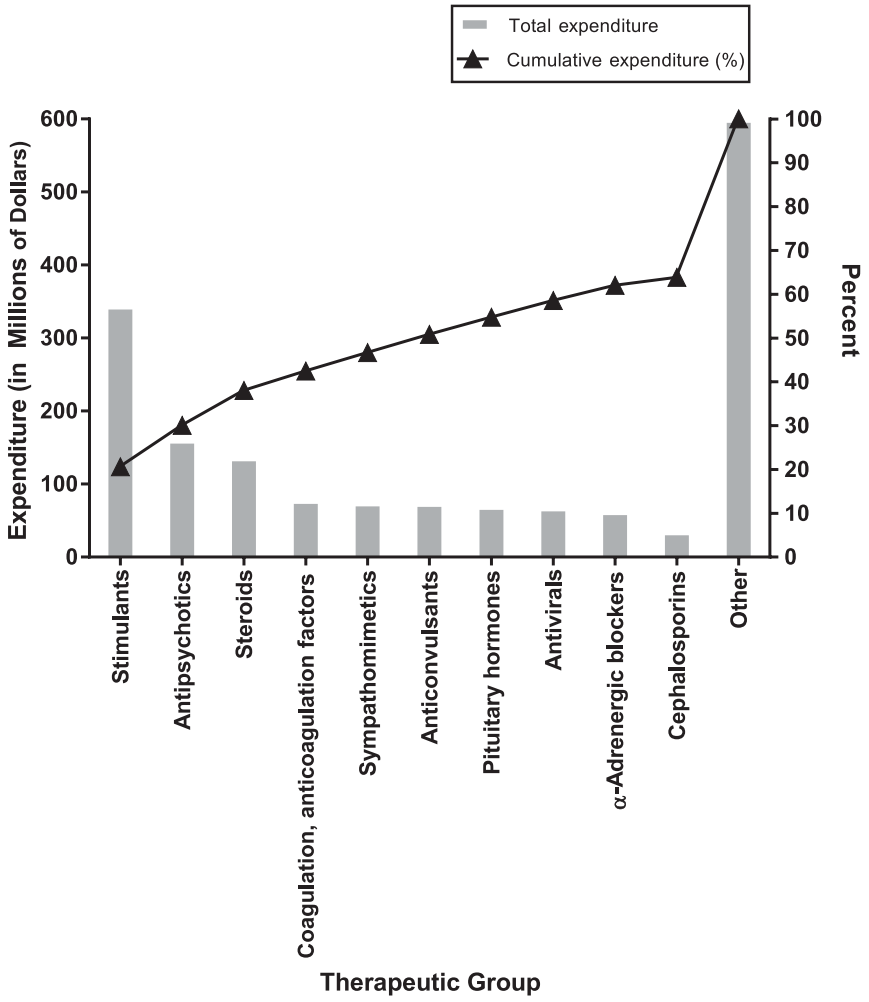

FIGURE 1
Pareto diagram of pharmaceutical expenditures by medication class.

Table 1. Overall, 1 311 199 children used 1 of the top 10 spending classes, accounting for 40.1% of the entire cohort. High-spending medication users were predominantly boys (54.3% vs 47.1% girls) and white (50.9% vs 40.6% of girls). They were more likely to have a disability eligibility criterion (8.2% vs 3.5% of girls), a chronic condition of any complexity (77.9% vs 41.6% of girls), a mental health condition (35.7% vs 11.9% of girls), and a complex chronic condition (9.8% vs 4.3% of girls) (all *P* values <.001). Median total health spending on users of these medications was more than 3 times that of other Medicaid enrollees (median interquartile range, $2153 [$1138–$4309] vs $699 [$284–$1430], *P* < .001), of which 24.1% was attributable to the top 10 medication classes. Ninety-two percent of the users of high-expenditure medications used more than 1 class of medication during 2013.

Use and Expenditure Within Each of the 10 Highest-Expenditure Medication Classes

Use varied widely among the classes, ranging from <0.1% (coagulation and anticoagulation factors) to 16.5% (inhaled steroids) of all enrollees (Table 2). Total health care spending was highest among users of sympathomimetic medications ($2.95 billion); this relatively large group (18% of all enrollees) had the lowest average health care spending per user ($5008). In contrast, the relatively small number of patients using coagulation and anticoagulation factors had the lowest overall spending ($88.8 million) but the highest aggregate spending per user ($86 511). Attribution of overall health care spending to the medication class ranged widely, from 1.4% (cephalosporins) to 82.2% (coagulation and anticoagulation factors). Similarly, attribution of any medication spending to overall health care spending varied from 17.3% (cephalosporins) to 84.8% (coagulation and anticoagulation factors).

Monotherapy (medication from 1 class only) was uncommon among all groups, ranging from 1.8% (α-adrenergic blockers) to 12.6% (stimulants). However, the type of medication classes used among those with polypharmacy varied across the groups. Over half (53.7%) of the children using antipsychotic agents also used stimulants (amphetamine-type), and almost half (49.3%) were using antidepressants during the year.

Specific Medications Within High-Expenditure Medication Classes

The 3 medications with the highest expenditures within each of the 10 high-expenditure medication classes are presented in Fig 3. The 4 highest-expenditure medications were all psychotropic medications (methylphenidate [stimulant, amphetamine-type]; 11.0% of all pharmaceutical expenditures), aripiprazole (antipsychotic; 9.2% of all expenditures), lisdexamfetamine (stimulant, amphetamine-type; 8.6% of all expenditures), and amphetamine salt combination (stimulant, amphetamine-type; 7.2% of all expenditures). Two of these medications (ariprirazole [Abilify and Bristol-Myers Squibb] and lisdexamfetamine [Vyvanse and Shire]) were patented

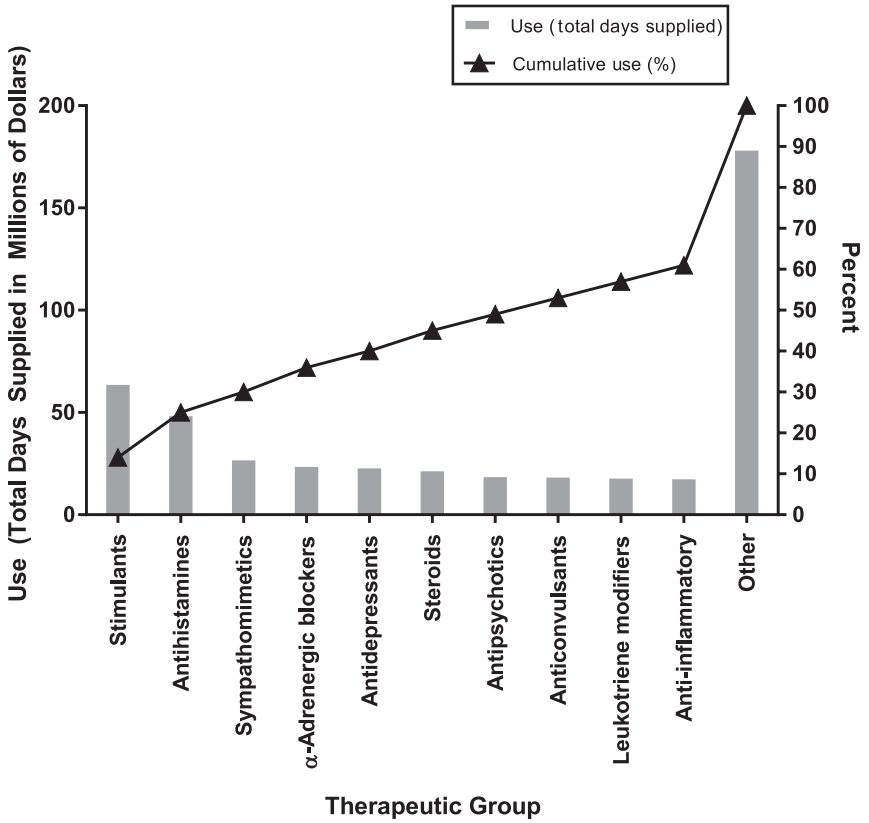

FIGURE 2
Pareto diagram of pharmaceutical use (total days supplied) by medication class.

single-source drugs without generic equivalents. The most commonly used individual medication in terms of the number of days supplied was the sympathomimetic albuterol, an inhaled β_2 agonist, but it only accounted for 4.3% of all drug expenditures. Among the anticoagulation and coagulation factors, antihemophilia factors each accounted for ≥1% of overall spending but only accounted for a small proportion (<0.1%) of use (in total days supplied). Similarly, among the pituitary hormones, somatotropin accounted for >5% of expenditure but comprised only 0.1% of use.

DISCUSSION

In a large, multistate Medicaid database, we found intense skewing of outpatient medication expenditures. These findings suggest that a relatively small number of medication classes account for a substantial proportion of pediatric pharmaceutical spending, with the top 10 medication classes accounting for more than half of that spending. Medications that account for the greatest proportion of spending are those likely to be used for the management of chronic mental health conditions (stimulants, antipsychotics, and α-adrenergic blockers [such as guanfacine and clonidine] that are typically used for behavioral conditions). Other high-expenditure medications included relatively inexpensive drugs used for high-prevalence conditions (eg, asthma) and costly medications used to treat relatively rare conditions (eg, hemophilia).

Other authors have described high-expenditure and high-use pediatric pharmaceuticals in terms of trends over time. One study of drug use from a large, multipayer prescription claims database from 2002 to 2010 included asthma and attention-deficit/hyperactivity disorder (ADHD) as 2 of the 3 most common indications for medications in children, but it did not include expenditure estimates.[21] An increase in ADHD drug use was described[22] but at a time when methylphenidate dominated the market. Our findings also showed a high impact of ADHD medications, but the greatest spending was on lisdexamfetamine, which may reflect ongoing changes in prescribing since 2010 and/or a differential prescription pattern specific to Medicaid enrollees.

A more recent analysis of 3 years of data on ~180 000 enrollees in California Children's Services, an insurance program for children with serious chronic conditions, found that 41% of expenditures were for hemophilia products used by just 0.4% of the cohort.[23] We also found high spending on hemophilia agents, but the predominance of behavioral and asthma medications overall in Medicaid (which do not meet the strict criteria for qualifying diagnoses in California Children's Services) and/or different prices for these medications in California may explain the relatively smaller effect of hemophilia treatment on overall drug spending in our cohort.

The study findings suggest potential targets for quality improvement in the care of high-cost pediatric patients in Medicaid. Previous research has focused on high users of hospital-based services for whom intensive care coordination may avert unnecessary hospitalizations and maximize community-based quality of life,[24] often through structured complex care programs.[25] A second target, more relevant to high-expenditure medication users, would focus on ensuring that enrollees are on appropriate evidence-based therapies both for common chronic conditions like mental and behavioral

TABLE 1 Characteristics of Children Using Versus Not Using at Least 1 Medication Within the 10 Medication Classes With the Highest Expenditures, 2013

Attributes	Overall	Children Using a High-Spending Medication	Children Not Using a High-Spending Medication	*P*[a]
N (%)	3 271 081	1 311 199 (40.1)	1 959 882 (59.9)	—
Age in years, *n* (%)				
0	35 609 (1.1)	13 051 (1)	22 558 (1.2)	<.001
1–4	790 267 (24.2)	341 362 (26)	448 905 (22.9)	—
5–12	1 446 956 (44.2)	594 912 (45.4)	852 044 (43.5)	—
13–18	998 249 (30.5)	361 874 (27.6)	636 375 (32.5)	—
Sex, *n* (%)				
Male	1 635 295 (50.0)	711 413 (54.3)	923 882 (47.1)	<.001
Female	1 635 786 (50.0)	599 786 (45.7)	1 036 000 (52.9)	—
Race/ethnicity, *n* (%)				
White	1 462 802 (44.7)	667 923 (50.9)	794 879 (40.6)	<.001
African American	1 130 065 (34.5)	398 341 (30.4)	731 724 (37.3)	—
Hispanic	274 495 (8.4)	87 779 (6.7)	186 716 (9.5)	—
Other	403 719 (12.3)	157 156 (12)	246 563 (12.6)	—
Basis of eligibility, *n* (%)				
Disability	177 011 (5.4)	107 877 (8.2)	69 134 (3.5)	<.001
Other	3 094 070 (94.6)	1 203 322 (91.8)	1 890 748 (96.5)	—
No. of chronic condition of any complexity,[b] *n* (%)				
0	1 433 736 (43.8)	290 099 (22.1)	1 143 637 (58.4)	<.001
1	1 021 927 (31.2)	471 809 (36)	550 118 (28.1)	—
2	494 341 (15.1)	310 304 (23.7)	184 037 (9.4)	—
≥3	321 077 (9.8)	238 987 (18.2)	82 090 (4.2)	—
Mental health condition, *n* (%)	700 356 (21.4)	467 707 (35.7)	232 449 (11.9)	<.001
No. of complex chronic conditions,[c] *n* (%)				
0	3 057 567 (93.5)	1 182 514 (90.2)	1 875 053 (95.7)	<.001
1	170 856 (5.2)	97 810 (7.5)	73 046 (3.7)	—
2	29 108 (0.9)	19 937 (1.5)	9171 (0.5)	—
≥3	13 550 (0.4)	10 938 (0.8)	2612 (0.1)	—
Total health care spending, $	9 642 933 898	6 659 809 971	2 983 123 927	—
Total health care spending per user[d] in $ (median interquartile range)	1121 (482–2507)	2153 (1138–4309)	699 (284–1430)	<.001
Spending attributable to top 10 medication classes (% overall spend)	16.6	24.1	—	—
No. drug classes used, *n* (%)				
0	894 865 (27.4)	—	894 865 (45.7)	<.001
1	498 565 (15.2)	104 485 (8)	394 080 (20.1)	—
2–3	817 360 (25)	369 650 (28.2)	447 710 (22.8)	—
4–5	514 247 (15.7)	359 107 (27.4)	155 140 (7.9)	—
≥6	546 044 (16.7)	477 957 (36.5)	68 087 (3.5)	—

—, not applicable.

[a] For comparisons between children using a high-spending medication versus children not using a high-spending medication.

[b] Based on the Agency for Healthcare Research and Quality Chronic Condition Indicator classification system.[18]

[c] Defined as those conditions lasting ≥12 months, involving either severe single-organ or multiorgan dysfunction, and were probably associated with hospitalization in a tertiary care center. These were ascertained by using an established set of ICD-9-CM codes.[19]

[d] Includes emergency department home health, inpatient, mental health, pharmacy outpatient, primary care, specialty outpatient care, and other services (eg, physical therapy).

health disorders and asthma, as well as more uncommon diseases like hemophilia and growth hormone deficiency.

The large contribution of spending on medications used to treat mental health-related conditions is particularly notable. Improvement targets in mental and behavioral health could include enhancing medication reconciliation, particularly when polypharmacy puts patients at higher-than-average risk of medication errors,[26,27] or providing access to greater behavioral health supports. Further study is needed comparing newer psychotropic medications (eg, aripriprazole and lisdexamfetamine) with older, potentially cheaper comparators (eg, risperidone and methylphenidate).

Growth in the use of psychotropic medications, particularly in young children, has received substantial media attention[28]; it is not known how much of this reflects physicians electing to prescribe pharmacotherapies (often multiple psychotropic medications in those receiving antipsychotics) when access to psychological or other supportive services is limited or lacking. Interventions implemented by states to strengthen oversight and limit prescribing of antipsychotics, such as instituting previous authorization processes[29] or telephone access for primary care providers to consult child psychiatrists,[30] may be contributing to recent reports of decreased rates of prescribing of antipsychotics in pediatric Medicaid enrollees.[31]

Study limitations include those related to the use of claims data. Although prescription data from such sources are generally thought to be reliable and valid,[32] we cannot track actual use (as distinct from dispensing) of medications. Given that we cannot link clinical indications or care to prescribing practices, inferences about the

TABLE 2 Use and Spending Within Each of the 10 Highest-Expenditure Medication Classes, 2013

	Stimulant, Amphetamine-Type	Antipsychotics	Inhaled Steroids	Coagulation, Anticoagulation Factors	Sympatho-mimetics	Anticonvulsants	Pituitary Hormones	Antivirals	α-Adrenergic Blockers	Antibiotics, Cephalosporins
Rank (spending)	1	2	3	4	5	6	7	8	9	10
Rank (use)[a]	1	7	6	115	3	8	34	36	5	19
Users,[b] *n* (% using)	265 970 (8.1)	75 129 (2.3)	540 737 (16.5)	1026 (<0.1)	588 936 (18.0)	72 308 (2.2)	15 489 (0.5)	135 798 (4.2)	109 399 (3.3)	417 330 (12.8)
Total use in days supplied, *n*	63 479 275	18 423 809	21 191 570	97 132	26 553 645	18 207 205	2 223 278	2 116 485	23 360 921	5 864 724
Total health care spending among users, $	1 715 606	1 178 840	2 808 342	88 761	2 949 108	1 368 546	239 681	834 430	1 193 584	2 154 049
Health care spending per user, $	6450	15 690	5194	86 511	5008	18 927	15 474	5967	10 910	5162
Spending attributable to medication class,[c] $ (% overall)	339 209 (19.8)	155 519 (13.2)	131 230 (4.7)	72 942 (82.2)	69 459 (2.4)	68 656 (5.0)	64 811 (27.0)	62 611 (7.5)	57 391 (4.8)	30 011 (1.4)
Spending attributable to any medication,[c] $ (% overall)	607 914 (35.4)	325 058 (27.6)	587 076 (20.9)	75 255 (84.8)	624 008 (21.2)	282 932 (20.7)	103 185 (43.1)	172 836 (20.7)	350 102 (29.3)	372 782 (17.3)
Number of drug classes used, *n* (%)										
1 (received drug in class only)	33 536 (12.6)	1454 (1.9)	10 818 (2.0)	100 (9.7)	21 098 (3.6)	2570 (3.6)	1168 (7.5)	10 958 (8.1)	2023 (1.8)	20 760 (5.0)
2–3	79 241 (29.8)	13 295 (17.7)	100 928 (18.7)	214 (20.9)	123 986 (21.1)	10 573 (14.6)	3477 (22.4)	31 942 (23.5)	25 701 (23.5)	90 080 (21.6)
4–5	64 240 (24.2)	18 382 (24.5)	150 378 (27.8)	213 (20.8)	163 854 (27.8)	14 718 (20.4)	3550 (22.9)	32 143 (23.7)	28 737 (26.3)	105 566 (25.3)
≥6	88 953 (33.4)	41 998 (55.9)	278 613 (51.5)	499 (48.6)	279 998 (47.5)	44 447 (61.5)	7294 (47.1)	60 755 (44.7)	52 938 (48.4)	200 924 (48.1)
Other medication classes most commonly used (%)	Antihistamines (31.2), α-adrenergic blockers (29.7)	Stimulant, (amphetamine-type) (53.7), antidepressants (49.3)	Sympatho-mimetics (62), penicillin antibiotics (51.4)	Penicillin antibiotics (35.9), analgesics (28.7)	Inhaled steroids (56.9), penicillin antibiotics (49)	Antidepressants (35.8), antihistamines (49.3)	Stimulants (amphetamine-type) (37.0), antihistamines (32.8)	Penicillin antibiotics (47.7), antihistamines (34.7)	Stimulants (amphetamine-type) (72.3), antihistamines (35.3)	Penicillin antibiotics (51.0), antihistamines (39.0)

[a] Use is defined as the total number of days of medication dispensed in 2013.

[b] Users are defined as the total number of enrollees with a prescription filled in 2013 among the 3 271 081 children in the data set.

[c] Among users of the medication class.

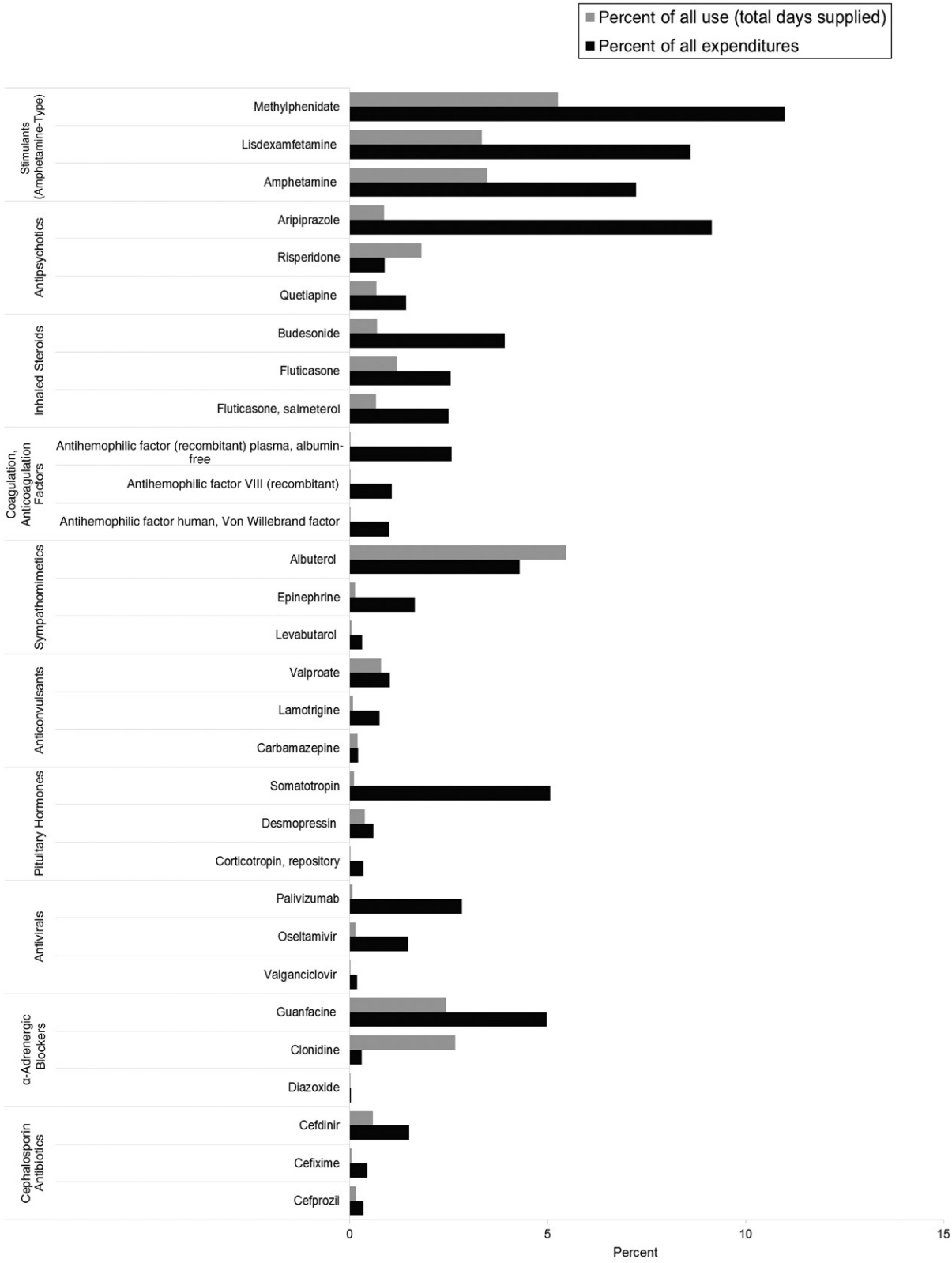

FIGURE 3

Expenditures (in dollars) and use (total days supplied) of individual medications (chemical moieties) with the highest expenditures. Individual medications from within the 10 medication classes with the highest expenditures are shown. Proportions of overall use and expenditures are based on 454.8 million days of medication use and $1.65 billion overall estimated spending on medications in the Medicaid Marketscan Database in 2013.

appropriateness of prescribing choices cannot be made; high use and expenditures of many medication classes may be indicative of high-quality care, although overprescribing within Medicaid may be occurring for some (eg, antipsychotic medications).[33] We were limited to drugs with an NDC and reimbursement; data on over-the-counter medications, herbal remedies, and supplements were lacking. Our expenditure estimates were extrapolated from use and an algorithm of spending estimation. We did not have data on actual payments made through Medicaid or by patients and families through copays, deductibles, or other out-of-pocket expenses. The use of payments to pharmacies inflates actual expenditures by state Medicaid programs, which are reduced via confidential rebates. Rebates for individual drugs can be high when there is significant competition between therapies from different manufacturers, and manufacturers must pay additional rebates to Medicaid if prices for medicines rise faster than inflation. Use and spending by individual states in the Truven database were not available, so interstate comparisons could not be made. Findings from Medicaid may also not be generalizable to privately insured patients, who are less likely to have chronic conditions and may have different use and spending patterns. Researchers in subsequent studies may assess differences in use and expenditures by type of insurance.

Deidentified data precluded state identification; patterns of drug use and expenditures may vary across states or in states not represented in the database. We also limited our analysis to a single year. Some medication use patterns may have changed in the ensuing years. For instance, findings may differ with the uptake of highly expensive, novel agents aimed at rare diseases, such as ivacaftor (Kalydeco) for cystic fibrosis and eculizumab (Soliris) for paroxysmal nocturnal hemoglobinuria and atypical hemolytic uremic syndrome.

CONCLUSIONS

Pediatric pharmaceutical expenditure under Medicaid is concentrated among medications prescribed for relatively common chronic health conditions, particularly mental health conditions and, to a lesser extent, among expensive drugs for serious, low-prevalence conditions, such as hemophilia. In this study, we highlight some potential priority medication classes and therapeutic areas of drugs for studies of comparative cost effectiveness and safety in children as well as high-value care initiatives to optimize the use of these medications.[34]

ACKNOWLEDGMENT

We thank Yuna Lee, BA, for assistance in preparing this article.

ABBREVIATIONS

ADHD: attention-deficit/hyperactivity disorder
NDC: National Drug Code

DOI: https://doi.org/10.1542/peds.2017-1095

Accepted for publication Jun 5, 2017

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PEDIATRICS (ISSN Numbers: Print, 0031-4005; Online, 1098-4275).

FINANCIAL DISCLOSURE: The authors have indicated they have no financial relationships relevant to this article to disclose.

FUNDING: Dr Cohen was supported by The Commonwealth Fund (a private, independent foundation based in New York City) and the Canadian Foundation for Healthcare Improvement as a 2015–2016 Harkness Fellow. Dr Berry was supported by the Agency for Healthcare Research and Quality (R21HS023092-01). The funders played no role in the design or conduct of the study; the collection, management, analysis, or interpretation of data; the preparation, review, or approval of the manuscript; or the decision to submit for publication. The views herein are those of the authors and not those of the funders, their directors, officers, or staff.

POTENTIAL CONFLICT OF INTEREST: Dr Cohen is a member of the Committee to Evaluate Drugs which provides advice to Ontario's Ministry of Health and Long-Term Care on public drug policy. This Canadian governmental ministry had no role in the design and conduct of the study; collection, management, analysis and interpretation of data; preparation, review, or approval of the manuscript; or the decision to submit for publication. The views herein are solely those of the authors; the other authors have indicated they have no potential conflicts of interest to disclose.

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