

# Assessment of botulinum toxin treatment in pediatric cerebral palsy patients in the United States managed Medicaid population

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## INTRODUCTION

- Botulinum toxin (BoNT) has been used to treat children with cerebral palsy (CwCP) and focal spasticity for more than 20 years and has become a recommended part of the cerebral palsy (CP) treatment armamentarium
- Little is published about the demographics, comorbidities and functional status and motivation for BoNT use in children, outside of clinical trials

### Brief History on BoNTs in CwCP

Year	Investigator	Findings
1993	Koman, et. al	Published the first study of BoNTs in 27 CwCP <sup>1</sup>
2006	Criswell, et. al	Proposed treatment algorithms for CwCP and lower limb spasticity <sup>2</sup>
2006	European Paediatric Neurology Society	Released consensus statements on the use of BoNT for CwCP, describing published data and best evidence and practices for BoNTs <sup>3,4</sup>
2009	European Journal of Neurology	Published an International Consensus Statement on the use of BoNTs for lower limb spasticity in CwCP <sup>5</sup>
2010	American Academy of Neurology	Released a practice parameter recommending that CwCP and localized/segmental spasticity be offered BoNTs <sup>6</sup>
2013	Novak, et. al	Published a systematic review of interventions for CwCP and classified the use of BoNTs as a "green light" intervention <sup>7</sup>
2016	Ipsen Biopharmaceuticals, Inc.	Dysport <sup>®</sup> is the first and only botulinum toxin type A granted FDA approval for the treatment of lower limb spasticity in children aged two and older <sup>8</sup>

## OBJECTIVES

- This analysis aims to quantify epidemiological estimates of CP in Medicaid managed care claims data, describe demographics of CwCP and explore characteristics of CwCP utilizing BoNT therapy

## STUDY DESIGN

### Starting data

- 2013-2015 Medicaid managed care enrollment and claims data from the Milliman Consolidated Health Cost Guidelines Sources Database (CHSD)
  - CHSD is a proprietary database of Medicaid managed care claims data contributed by health plans in the United States (US)
- CHSD includes over 17 million enrolled members over years 2013-2015 and represents 16+ states in the US

### Established the denominator population

- The following were excluded from the denominator population:
  - Some members (<1%) due to data quality issues, members not aged 2 to 20 years, all members in various states due to data quality issues and low enrollment rates (15 states remained), and children with <11 months of enrollment in a year
- Defined a child year (CY) as one year of time of children aged 2 to 20 with ≥11 months of enrollment in a year

### Identified CwCP within denominator population

- By ICD-9 (infantile cerebral palsy: 343.x, athetoid cerebral palsy: 333.71) and/or ICD-10 (cerebral palsy: G80.x) diagnosis codes
- Must have ≥1 inpatient or observation claim with a CP diagnosis code, or 2 emergency department, non-acute inpatient, or outpatient claims with CP diagnosis codes on separate days, over a 3 year period, inclusive of years with <11 months

### Evaluated characteristics of CwCP for each CY

- Sex
- Age groups: 2-3, 4-8, 9-13, 14-18, 19-20
- US Regions: North West (AK, ID, MT, OR, WA, WY), South West (AZ, CA, HI, NV, UT), North Central (IA, IL, IN, MI, MN, ND, NE, OH, SD, WI), South Central (AR, CO, KS, LA, MO, NM, OK, TX), North East (CT, DE, DC, MA, MD, ME, NH, NJ, NY, PA), and South East (AL, FL, GA, KY, MS, NC, SC, TN, VA, WV)
- BoNT use and use in the previous year (unknown for 2013 CYs)
- Comorbidities defined by the Health & Human Services Hierarchical Condition Categories (HHS-HCCs)<sup>9</sup> based on claims diagnoses during the 3 year period. CCs were aggregated (Table 1)
- Medical equipment & supplies (MES) use. The MES in Figure 3 account for most MES for CwCP and could be indicative of functional status

Table 1. Condition Classifications (CCs) Reported in this Study

CC	CC Name	Aggregation
CC096	Prader-Willi, Patau, Edwards, and Autosomal Deletion Syndromes	Intellectual Disability
CC097	Down Syndrome, Fragile X, Other Chromosomal Anomalies, and Congenital Malformation Syndromes	
CC102	Autistic Disorder	
CC103	Pervasive Developmental Disorders, Except Autistic Disorder	
CC130	Congestive Heart Failure	Heart Defects and Disease
CC138	Major Congenital Heart/Circulatory Disorders	
CC139	Atrial and Ventricular Septal Defects, Patent Ductus Arteriosus, and Other Congenital Heart/Circulation	
CC142	Specified Heart Arrhythmias	
CC160	Chronic Obstructive Pulmonary Disease, Including Bronchiectasis	Asthma and Lung Disease
CC161	Asthma	
CC162	Fibrosis of Lung and Other Lung Disorders	none
CC023	Protein-Calorie Malnutrition	
CC112	Quadriplegic Cerebral Palsy	
CC120	Seizure Disorders and Convulsions	
CC121	Hydrocephalus	
CC122	Non-Traumatic Coma, Brain Compression/Anoxic Damage	
CC125	Respirator Dependence/Tracheostomy Status	

Note: CCs were developed for the Centers for Medicaid & Medicare Services (CMS) and are used as the starting point for HCC-CMS risk adjustment. CMS has determined that children with these CCs have higher medical costs than children without these CCs. There are 119 child CCs used for risk adjustment. We reported the most common CCs for CwCP.

### Calculated Relative Risk (RR) statistics

$$RR \text{ Ratio} = \frac{CYs \text{ with characteristic and with BoNT} / \text{Total } CYs \text{ with characteristic}}{CYs \text{ without characteristic and with BoNT} / \text{Total } CYs \text{ without characteristic}}$$

- Relative risk p-values, with significance attributed to p-values < 0.05

Figure 1A. Percent of CwCP by Sex

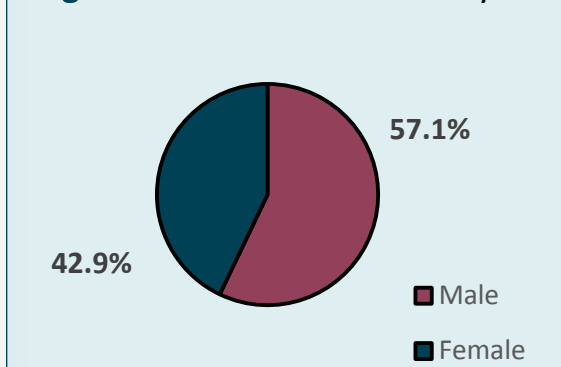
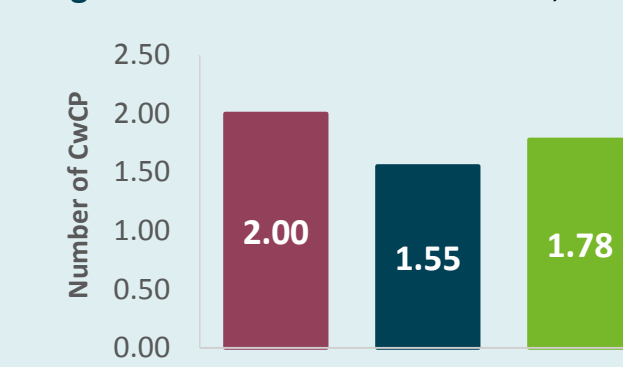


Figure 1B. CwCP Prevalence Per 1,000



## RESULTS

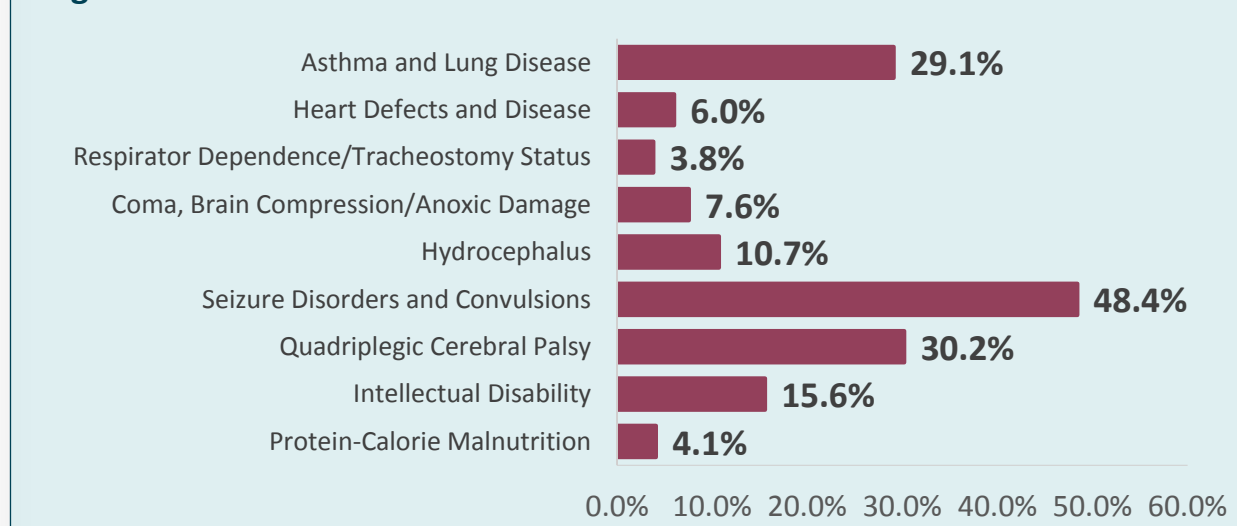
### CwCP prevalence

- The prevalence of CP in Medicaid claims data was 1.78 per 1,000 children (Figure 1B)
  - 7,158 CwCP CYs of 4,022,173 denominator CYs
- This rate is lower than what is presented in the literature, possibly because some state Medicaid plans exempt disabled children from managed care enrollment<sup>10</sup>
- 57.1% male and 42.9% female; male prevalence was 2 per 1,000 children and female prevalence was 1.55 per 1,000 children (Figures 1A, Figure 1B)

### Comorbidities and quadriplegic CP

- 30.2% of CwCP were specifically diagnosed with quadriplegic CP
- Of the CCs reported, seizure disorder or convulsions was the most common (48.4%) in CwCP
- Other comorbidities included asthma and lung disease (29.1%), intellectual disability (15.6%), hydrocephalus (10.7%) (Figure 2)
- We believe CwCP with intellectual disabilities are underrepresented as there is rarely reason to include the diagnosis on a claim

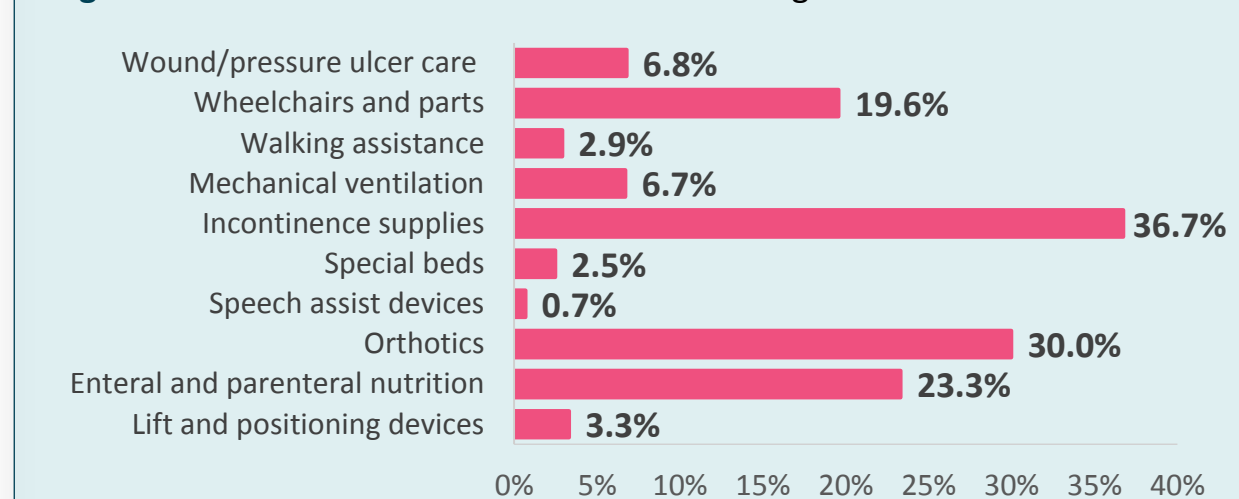
Figure 2. Percent of CwCP with Select Comorbidities



### Medical equipment & supplies use

- The most often utilized MES included incontinence supplies (36.7%), orthotics (30.0%), enteral and parenteral nutrition (23.3%), wheelchairs and parts (19.6%), and mechanical ventilation (6.7%) (Figure 3)

Figure 3. Percent of CwCP with MES Claims during CY



### BoNT treatment in CwCP

- 9.4% of CwCP had BoNT treatment during a year
- Children aged 4-8 years were most likely to have BoNTs (13.2%) and older children ages 14-18 and 19-20 were the least likely (6.9% and 3.7%, respectively) (Figure 4)
- BoNT use varied by region and was most common in the North East (13.8%) and the North West (11.9%) (Figure 5)
- 60.6% of CwCP receiving BoNT in a year had BoNT the next year
- CwCP diagnosed with coma and/or brain compression/anoxic damage were most likely to receive BoNTs (17.9%), followed by those with quadriplegic CP (17.5%)
- CwCP diagnosed with intellectual disabilities were least likely to receive BoNTs (5.1%) (Figure 6)
- Use of MES was associated with higher rates of toxin use (however, one was not statistically significant) (Figure 7)

Figure 4. Percent of CwCP with BoNT during CY, by Age Group

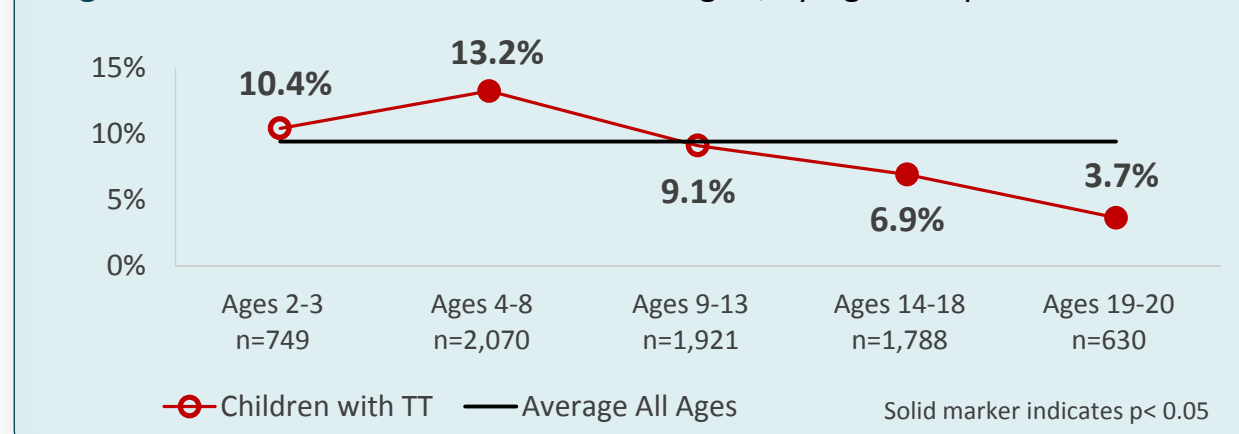


Figure 5. Percent of CwCP with BoNT during CY, by Region

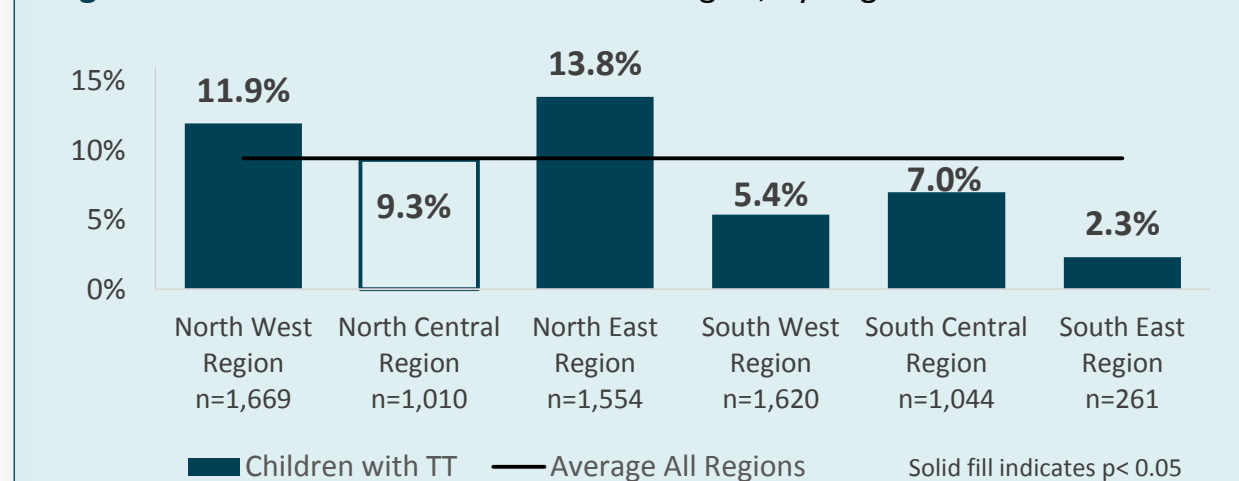


Figure 6. Percent of CwCP with BoNT during CY, by Comorbidities

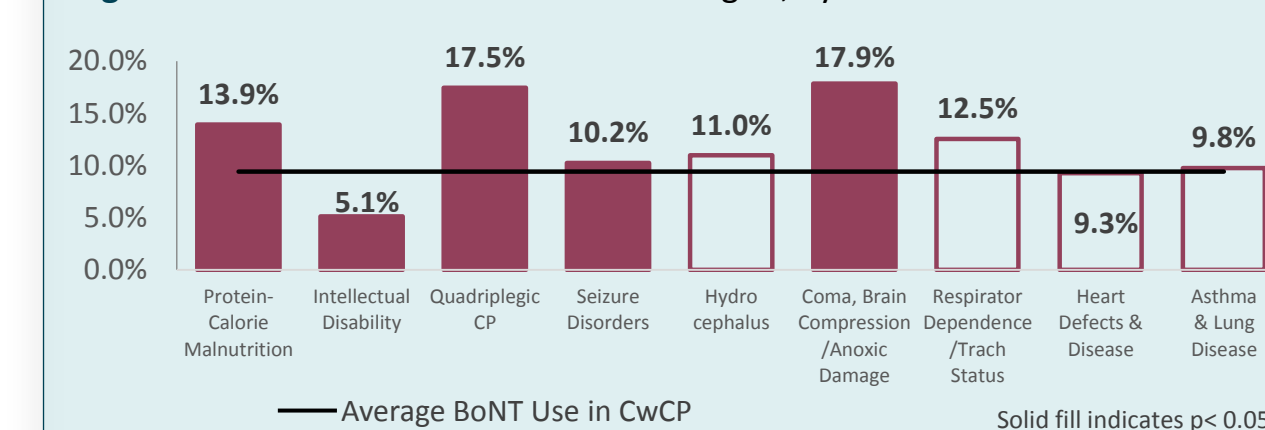
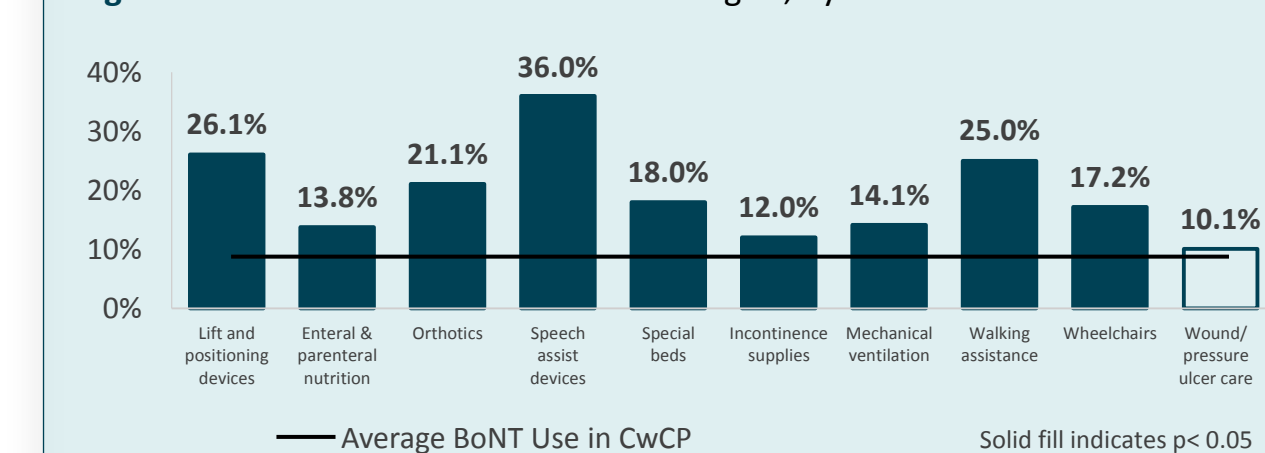


Figure 7. Percent of CwCP with BoNT during CY, by MES Use



## LIMITATIONS

This analysis relies on managed Medicaid claims data from a subset of managed care organizations in only 15 US states. The data may not be representative of the entire US or a particular region or state, and not all Medicaid CwCP are enrolled in managed care. There are substantial variations in state Medicaid policies, managed care benefits and payment policies. Claims may not accurately reflect all of the diagnoses present in each patient.

## CONCLUSIONS

- The prevalence estimate of cerebral palsy this study was low compared to rates from previously published studies
- Usage of BoNTs varied significantly throughout the US regions
- Among all age groups, younger CwCP had the highest rates of BoNT treatment
- Both, CwCP with quadriplegia and CwCP with selected MES had higher BoNT use than overall CwCP, suggesting that in addition to facilitating mobility, BoNTs may be used for palliative relief and care management
- Further research should be conducted to understand treatment patterns, costs and outcomes of CwCP

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