**Supplemental Table 1. CPT Codes, Cumulative Frequencies, and Unit Costs in 2018 US dollars for Selected Elements from the 2018 DMD Care Considerations.**

| **2018 DMD Care Considerations Key Elements** | **CPT** **Code (modifier)\*** | **Annual**  **Frequency†** | **Cumulative Frequency‡** | **Median Unit Cost in US $**§ | **Expected Out-of-Pocket Payment in US $** |
| --- | --- | --- | --- | --- | --- |
| **Neuromuscular Management** | | | | |  |
| Neuromuscular Specialist£ | 99215 | Semiannual | 40 | 213 | 25 |
| Range of motion Testing | 95851 (59) | Semiannual | 40 | 93 | 25 |
| Performance Testing | 97750 (GP) | Semiannual | 40 | 53 | 0 |
| Brief mental health assessment | 96127 | Semiannual | 40 | 13 | 25 |
| **Rehabilitation Management** | | | | |  |
| Physical Therapist£ | 97002 (GP) | Semiannual | 40 | 61 | 0 |
| Occupational Therapist£ | 97004 (GO) | Semiannual | 40 | 58 | 0 |
| Speech Therapist | na# | As needed | 2 | 86 | 15 |
| Evaluation of speech sound production | 92506/92522 | As needed | 1 | 101 | 15 |
| Diagnostic swallowing test (Motion fluoroscopy/swallow) | 92611/72430 (26) | As needed | 1 | 94 | 25 |
| **Respiratory Management** | | | | |  |
| Pulmonary Specialist£ | 99215 | Annual < LOA; Semiannual ≥ LOA | 34 | 213 | 25 |
| Forced Vital Capacity | 94010 | Annual < LOA; Semiannual > LOA | 34 | 70 | 0 |
| Maximum insufflation pressure/Maximum exsufflation pressure | 94200 | Semiannual ≥ LOA | 28 | 55 | 0 |
| Oximetry | 94760 | Semiannual ≥ LOA | 28 | 7 | 0 |
| Blood gases measurement | 82803 | Semiannual ≥ LOA | 28 | 22 | 0 |
| Polysomnography with technician present | 95810 | As needed < LOA; Annual ≥ LOA | 16 | 985 | 34 |
| **Cardiac Management** | | | | |  |
| Cardiac Specialist£ | 99215 | Annual | 20 | 213 | 20 |
| Electrocardiogram | 93000 (59) | Annual | 20 | 30 | 0 |
| Echocardiogram | 93306 | From diagnosis to < 7 | 2 | 465 | 30 |
| Cardiac Magnetic Resonance Imaging | 75557 (26)/  75561 (TC/26) | Annual ≥ 7 | 18 | 1312 | 244 |
| Holter monitoringΩ | 93224 | Annual after onset left ventricular dysfunction | 10 | 180 | 30 |
| **Orthopedic and Bone Heath Management** | | | | |  |
| Orthopedic Specialist£ | 99215 | Annual < LOA; Semiannual ≥ LOA | 34 | 213 | 22 |
| Vitamin D | 82652 | Annual | 20 | 37 | 0 |
| Dual-energy x-ray absorptiometry | 77080 | Annual | 20 | 92 | 15 |
| Lateral spine x-rays | 72020 | Annual | 20 | 34 | 0 |
| Spinal study | 72090/72081 | Annual < LOA; Semiannual ≥ LOA until corrective surgery | 14 | 86 | 25 |
| **Gastrointestinal and Nutritional Management** | | | | |  |
| Gastroenterology£ | 99215 | Semiannual | 40 | 220 | 20 |
| Dietician£ | 97802/97803 | Semiannual | 40 | 41 | 0 |

na=claims database not used to determine costs; CPT=Current Procedural Terminology; DMD=Duchenne muscular dystrophy; LOA=loss of ambulation; TC=technical component; GN=Services delivered under an outpatient speech-language pathology plan of care; GO=Services delivered under an outpatient occupational therapy plan of care; or GP**=**Services delivered under an outpatient physical therapy plan of care; US $=United States dollars.

**\***CPT codes were used to extract charges, unless otherwise noted. Multiple codes are included where multiple relevant CPT codes were identified. Modifiers were included when the majority of submitted charges included the modifier.

**†**Annual=1 time per year. As needed=minimum of 1. Biennial=every other year. Semiannual=2 times per year.

**‡**Calculated from recommendations of the 2018 DMD Care Considerations using a 20-year follow-up period and estimated from a diagnosis at age 5 years, loss of independent ambulation at 11 years, onset of left ventricular dysfunction at 15 years, and death at age 25.

§Median charges using a de-identified fully-insured insurance claims data from a large midwestern commercial health insurer.

£Established patient, 40 minutes

ΩHolter monitoring initiated at age 15 years per average age at onset of cardiac dysfunction reported by: Barber, B. J., J. G. Andrews, Z. Lu, N. A. West, F. J. Meaney, E. T. Price, A. Gray, D. W. Sheehan, S. Pandya, M. Yang and C. Cunniff (2013). "Oral corticosteroids and onset of cardiomyopathy in Duchenne muscular dystrophy." J Pediatr **163**(4): 1080-1084 e1081.

#Speech therapist identified by provider type assigned in claims dataset.

€Referral to an endocrinologist for growth failure or pubertal delay by age 14 years. Assume annual follow-up for monitoring treatment. Growth failure estimated to begin at age 11 per Ward, L. M. and D. R. Weber (2019). "Growth, pubertal development, and skeletal health in boys with Duchenne Muscular Dystrophy." Curr Opin Endocrinol Diabetes Obes **26**(1): 39-48.

**Supplemental Table 2. Selected Prescription Medications and Interventions, Costs in 2018 US dollars.**

| **Intervention** | **Follow-up Cost in US $** | **Expected Out-of-Pocket Payment in US $** |
| --- | --- | --- |
| Corticosteroids - prednisone\* | 894 | 789 |
| Corticosteroids - deflazacort\* | 2.1 million | 17,340 |
| Prophylactic cardiac medication\* | 60 | 41 |
| Mechanical insufflation-exsufflation device α | 4750 | na |
| Respiratory assist with bi-level pressure capability, non-invasive α | 15,600 | na |
| Spinal fusion¥ | 57,000 | na |

na=not available; US $=United States dollars.

\*Initiated at age 8 years through age 24 years (see Supplemental Tables 3-5 for calculations). Expected out-of-pocket expenses were calculated from percentage of direct cost paid by insured (prednisone=88%, lisinopril=84%) or $85 per 30-day prescription per special formulary (deflazacort).

αMechanical insufflation-exsufflation device cost from Bach, J. R., J. Tran and S. Durante (2015). "Cost and physician effort analysis of invasive vs. noninvasive respiratory management of Duchenne muscular dystrophy." Am J Phys Med Rehabil **94**(6): 474-482. Bi-level respiratory assist machine and supplies beginning at age 10 per unpublished MD STAR*net* surveillance data; cost of machine [E0471] plus supply repurchasing (HCPCS Codes: mask [A7034]; hose [A7037]; heated tubing [A4604]; disposable filter [ A7038]; headgear [A7035]; chinstrap [A7036]; heated humidifier [E0562].

¥Surgery cost minus readmission costs from Malik, A. T., E. Yu, J. Kim and S. N. Khan (2019). "Understanding costs in a 90-day episode of care following posterior spinal fusions for adolescent idiopathic scoliosis." World Neurosurg **130**: e535-e541.

**Supplemental Table 3. Unit Costs per 6-month Period in 2018 US Dollars for Prednisone.**

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
| **Age, y (half-year intervals)** | **Mean Weight (kg)\*** | **Dose**  **(0.75 mg/kg/day)** | **Price**  **(mg/kg/day)†** | **Price per half-year in US $** |
| 8.0-8.49 | 39.1 | 29.33 | 0.09 | 17 |
| 8.5-8.99 | 39.1 | 29.33 | 0.09 | 17 |
| 9.0-9.49 | 40.0 | 30.00 | 0.09 | 17 |
| 9.5-9.99 | 44.3 | 33.23 | 0.10 | 19 |
| 10.0-10.49 | 44.8 | 33.60 | 0.11 | 19 |
| 10.5-10.99 | 45.8 | 34.35 | 0.11 | 20 |
| 11.0-11.49 | 51.0 | 38.25 | 0.12 | 22 |
| 11.5-11.99 | 52.7 | 39.53 | 0.12 | 23 |
| 12.0-12.49 | 52.7 | 39.53 | 0.12 | 23 |
| 12.5-12.99 | 53.5 | 40.13 | 0.13 | 23 |
| 13.0-13.49 | 56.6 | 42.45 | 0.13 | 24 |
| 13.5-13.99 | 57.6 | 43.20 | 0.14 | 25 |
| 14.0-14.49 | 59.9 | 44.93 | 0.14 | 26 |
| 14.5-14.99 | 63.4 | 47.55 | 0.15 | 27 |
| 15.0-15.49 | 69.3 | 51.98 | 0.16 | 30 |
| 15.5-15.99 | 65.1 | 48.83 | 0.15 | 28 |
| 16.0-16.49 | 68.0 | 51.00 | 0.16 | 29 |
| 16.5-16.99 | 68.1 | 51.08 | 0.16 | 29 |
| 17.0-17.49 | 67.3 | 50.48 | 0.16 | 29 |
| 17.5-17.99 | 71.9 | 53.93 | 0.17 | 31 |
| 18.0-18.49 | 72.5 | 54.38 | 0.17 | 31 |
| 18.5-18.99 | 74.8 | 56.10 | 0.18 | 32 |
| 19.0-19.49 | 69.1 | 51.83 | 0.16 | 30 |
| 19.5-19.99 | 72.5 | 54.38 | 0.17 | 31 |
| 20.0-20.49 | 76.5 | 57.38 | 0.18 | 33 |
| 20.5-20.99 | 72.6 | 54.45 | 0.17 | 31 |
| 21.0-21.49 | 78.2 | 58.65 | 0.18 | 34 |
| 21.5-21.99 | 67.0 | 50.25 | 0.16 | 29 |
| 22.0-22.49\* | 63.3 | 47.48 | 0.15 | 27 |
| 22.5-22.99 | 63.3 | 47.48 | 0.15 | 27 |
| 23.0-23.49 | 63.3 | 47.48 | 0.15 | 27 |
| 23.5-23.99 | 63.3 | 47.48 | 0.15 | 27 |
| 23.5-23.99 | 63.3 | 47.48 | 0.15 | 27 |
| 23.5-23.99 | 63.3 | 47.48 | 0.15 | 27 |

mg=milligram; kg=kilogram; US $=United States dollars.

\*Weights from age 8.5 years through 22.49 years from Lamb, M. M., B. Cai, J. Royer, S. Pandya, A. Soim, R. Valdez, C. DiGuiseppi, K. James, N. Whitehead, H. Peay, S. Y. Venkatesh, D. Matthews, R. Muscular Dystrophy Surveillance and N. Tracking (2018). "The effect of steroid treatment on weight in non-ambulatory males with Duchenne muscular dystrophy." Am J Med Genet A **176**(11): 2350-2358.

**†**National Drug Code ID: 0603-5338-21. Price per pill ($0.03) calculated from package cost for 10 mg prednisone ($3.15/100 pills per package).

**Supplemental Table 4. Unit Costs per 6-month Period in 2018 US Dollars for Deflazacort.**

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
| **Age, y (half-year intervals)** | **Mean Weight (kg)\*** | **Dose**  **(0.90 mg per kg)** | **Price**  **(mg/kg/day) in US $†** | **Price per half-year (100% wholesale) in US $** |
| 8.0-8.49 | 39.1 | 35.19 | 218 | 39,753 |
| 8.5-8.99 | 39.1 | 35.19 | 218 | 39,753 |
| 9.0-9.49 | 40.0 | 36.00 | 223 | 40,668 |
| 9.5-9.99 | 44.3 | 39.87 | 247 | 45,040 |
| 10.0-10.49 | 44.8 | 40.32 | 250 | 45,548 |
| 10.5-10.99 | 45.8 | 41.22 | 255 | 46,565 |
| 11.0-11.49 | 51.0 | 45.90 | 284 | 51,852 |
| 11.5-11.99 | 52.7 | 47.43 | 294 | 53,580 |
| 12.0-12.49 | 52.7 | 47.43 | 294 | 53,580 |
| 12.5-12.99 | 53.5 | 48.15 | 298 | 54,394 |
| 13.0-13.49 | 56.6 | 50.94 | 315 | 57,546 |
| 13.5-13.99 | 57.6 | 51.84 | 321 | 58,562 |
| 14.0-14.49 | 59.9 | 53.91 | 334 | 60,901 |
| 14.5-14.99 | 63.4 | 57.06 | 353 | 64,459 |
| 15.0-15.49 | 69.3 | 62.37 | 386 | 70,458 |
| 15.5-15.99 | 65.1 | 58.59 | 363 | 66,188 |
| 16.0-16.49 | 68.0 | 61.20 | 379 | 69,136 |
| 16.5-16.99 | 68.1 | 61.29 | 379 | 69,238 |
| 17.0-17.49 | 67.3 | 60.57 | 375 | 68,424 |
| 17.5-17.99 | 71.9 | 64.71 | 401 | 73,101 |
| 18.0-18.49 | 72.5 | 65.25 | 404 | 73,711 |
| 18.5-18.99 | 74.8 | 67.32 | 417 | 76,050 |
| 19.0-19.49 | 69.1 | 62.19 | 385 | 70,254 |
| 19.5-19.99 | 72.5 | 65.25 | 404 | 73,711 |
| 20.0-20.49 | 76.5 | 68.85 | 426 | 77,778 |
| 20.5-20.99 | 72.6 | 65.34 | 404 | 73,813 |
| 21.0-21.49 | 78.2 | 70.38 | 436 | 79,507 |
| 21.5-21.99 | 67.0 | 60.30 | 373 | 68,119 |
| 22.0-22.49\* | 63.3 | 56.97 | 353 | 64,358 |
| 22.5-22.99 | 63.3 | 56.97 | 353 | 64,358 |
| 23.0-23.49 | 63.3 | 56.97 | 353 | 64,358 |
| 23.5-23.99 | 63.3 | 56.97 | 353 | 64,358 |
| 24.0-24.49 | 63.3 | 56.97 | 353 | 64,358 |
| 24.5-24.99 | 63.3 | 56.97 | 353 | 64,358 |

mg=milligram; kg=kilogram; US $=United States dollars.

\*Weights from age 8.5 years through 22.49 years from Lamb, M. M., B. Cai, J. Royer, S. Pandya, A. Soim, R. Valdez, C. DiGuiseppi, K. James, N. Whitehead, H. Peay, S. Y. Venkatesh, D. Matthews, R. Muscular Dystrophy Surveillance and N. Tracking (2018). "The effect of steroid treatment on weight in nonambulatory males with Duchenne muscular dystrophy." Am J Med Genet A **176**(11): 2350-2358.

**†**Cost estimate ($6.19 mg/kg) from Lin G.A., Koola C, Walton S, Agboola F, Seider M, Boyer N, Fluetsch N, Chapman R, Quach D, Kumar V.M., Rind D, Samur S, Pearson S. Deflazacort, Eteplirsen, and Golodirsen for Duchenne Muscular Dystrophy: Effectiveness and Value. Final Evidence Report. https://icer-review.org/wp-content/uploads/2018/12/ICER\_DMD-Final-Report\_081519.pdf: Institute for Clinical and Economic Review;August 15, 2019. November 24, 2021.

**Supplemental Table 5. Unit Costs per 6-month Period in 2018 US Dollars for Lisinopril.**

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
| **Age, y** | **Mean Weight (kg)\*** | **Dose (mg)†** | **Price per**  **Day in US $§** | **Price per half-year in US $** |
| 8.0-8.49 | 39.1 | 2.5 | 0.005 | 0.92 |
| 8.5-8.99 | 39.1 | 2.5 | 0.005 | 0.92 |
| 9.0-9.49 | 40.0 | 5.0 | 0.01 | 1.84 |
| 9.5-9.99 | 44.3 | 5.0 | 0.01 | 1.84 |
| 10.0-10.49 | 44.8 | 5.0 | 0.01 | 1.84 |
| 10.5-10.99 | 45.8 | 5.0 | 0.01 | 1.84 |
| 11.0-11.49 | 51.0 | 5.0 | 0.01 | 1.84 |
| 11.5-11.99 | 52.7 | 5.0 | 0.01 | 1.84 |
| 12.0-12.49 | 52.7 | 5.0 | 0.01 | 1.84 |
| 12.5-12.99 | 53.5 | 5.0 | 0.01 | 1.84 |
| 13.0-13.49 | 56.6 | 5.0 | 0.01 | 1.84 |
| 13.5-13.99 | 57.6 | 5.0 | 0.01 | 1.84 |
| 14.0-14.49 | 59.9 | 5.0 | 0.01 | 1.84 |
| 14.5-14.99 | 63.4 | 5.0 | 0.01 | 1.84 |
| 15.0-15.49 | 69.3 | 5.0 | 0.01 | 1.84 |
| 15.5-15.99 | 65.1 | 5.0 | 0.01 | 1.84 |
| 16.0-16.49 | 68.0 | 5.0 | 0.01 | 1.84 |
| 16.5-16.99 | 68.1 | 5.0 | 0.01 | 1.84 |
| 17.0-17.49 | 67.3 | 5.0 | 0.01 | 1.84 |
| 17.5-17.99 | 71.9 | 5.0 | 0.01 | 1.84 |
| 18.0-18.49 | 72.5 | 5.0 | 0.01 | 1.84 |
| 18.5-18.99 | 74.8 | 5.0 | 0.01 | 1.84 |
| 19.0-19.49 | 69.1 | 5.0 | 0.01 | 1.84 |
| 19.5-19.99 | 72.5 | 5.0 | 0.01 | 1.84 |
| 20.0-20.49 | 76.5 | 5.0 | 0.01 | 1.84 |
| 20.5-20.99 | 72.6 | 5.0 | 0.01 | 1.84 |
| 21.0-21.49 | 78.2 | 5.0 | 0.01 | 1.84 |
| 21.5-21.99 | 67.0 | 5.0 | 0.01 | 1.84 |
| 22.0-22.49 | 63.3 | 5.0 | 0.01 | 1.84 |
| 23.0-23.49 | 63.3 | 5.0 | 0.01 | 1.84 |
| 23.5-23.99 | 63.3 | 5.0 | 0.01 | 1.84 |
| 24.0-24.49 | 63.3 | 5.0 | 0.01 | 1.84 |
| 24.5-24.99 | 63.3 | 5.0 | 0.01 | 1.84 |

mg=milligram; kg=kilogram; US $=United States dollars.

\*Mean weights for males on corticosteroids from Lamb, M. M., B. Cai, J. Royer, S. Pandya, A. Soim, R. Valdez, C. DiGuiseppi, K. James, N. Whitehead, H. Peay, S. Y. Venkatesh, D. Matthews, R. Muscular Dystrophy Surveillance and N. Tracking (2018). "The effect of steroid treatment on weight in nonambulatory males with Duchenne muscular dystrophy." Am J Med Genet A **176**(11): 2350-2358.

**†**Dosage (2.5 mg/day for up to 40 kg, 5 mg/day for 40 kg and over) from**:** Viollet L, Thrush PT, Flanigan KM, Mendell JR, Allen HD. Effects of angiotensin-converting enzyme inhibitors and/or beta blockers on the cardiomyopathy in Duchenne muscular dystrophy. Am J Cardiol 2012;110(1):98-102.

§National Drug Code ID: 51672-4037-01. Price per pill ($0.01) calculated from package cost for 5 mg lisinopril ($1.02/100 pills per package).

**Supplemental Box 1. Examples of Estimating Costs in 2018 US Dollars**

**Example: Respiratory specialist visits**

Lifetime cost of respiratory specialist visits (from age 5 to age 25) = (($213 per visit x 1 visit per year) x 6 years) + (($213 per visit x 2 visits per year) x 14 years) + 0 = $7242

**Example: Cumulative cost respiratory management domain**

Pulmonology visits = (($213 x 1) x 6) + (($213 x 2) x 14) + 0 = $7242

Measurement of forced vital capacity = (($70 x 1) x 6) + (($70 x 2) x 14) + 0 = $2380

Measurement of maximum insufflation/exsufflation pressures = (($55 x 0) x 6) + (($55 x 2) x 14) + 0 = $1540

Oximetry = (($7 x 0) x 6) + ((($7 x 2) x 14) + 0 = $196

Measurement of blood gases = ((22 x 0) x 6) + ((($2 x 2) x 14) + 0 = $616

Mechanical In-Exsufflation device = ((0 x 0) x 0) + (((0 x 0) x 0) + $4750 = $4750

Continuous positive pressure device = ((0 x 0) x 0) + (((0 x 0) x 0) + $10850 = $10850

Polysomnography = (($985 x 0) x 2) + ((($985 x 1) x 14) + 0 = $15760

Total management domain cost = $7242 + $2380 + $1540 + $196 + $616 + $4750 + $10850 + $15760

Total cumulative charge=Neuromuscular + Rehabilitation + Respiratory + Cardiac + Orthopedic and Bone Health + Gastrointestinal and Nutritional + Endocrine