ANALYSIS OF CORTICOSTEROID USE IN AMBULATORY AND NONAMBULATORY MALES WITH DUCHENNE OR BECKER MUSCULAR DYSTROPHY WHO ENROLLED IN THE DUCHENNE REGISTRY

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BACKGROUND & OBJECTIVES

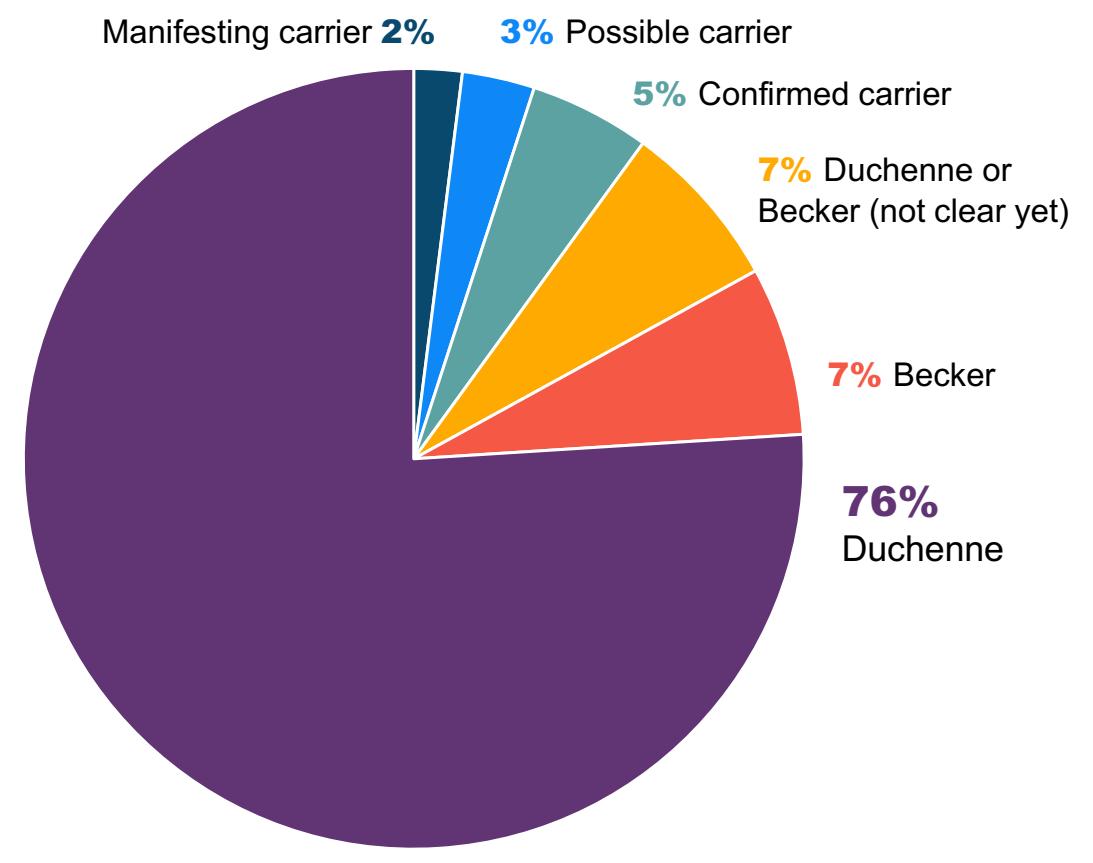
BACKGROUND ON THE DUCHENNE REGISTRY

General info

- Established by PPMD in 2007
- Largest U.S. registry for Duchenne
- Data collected via Duchenneregistry.org

Aims of collecting data

- Connecting individuals and families with actively recruiting trials
- Understanding current practice
- Feasibility for studies; understanding recruitment
- Protocol design (Inclusion / Exclusion)



The Duchenne Registry 10 Year Report

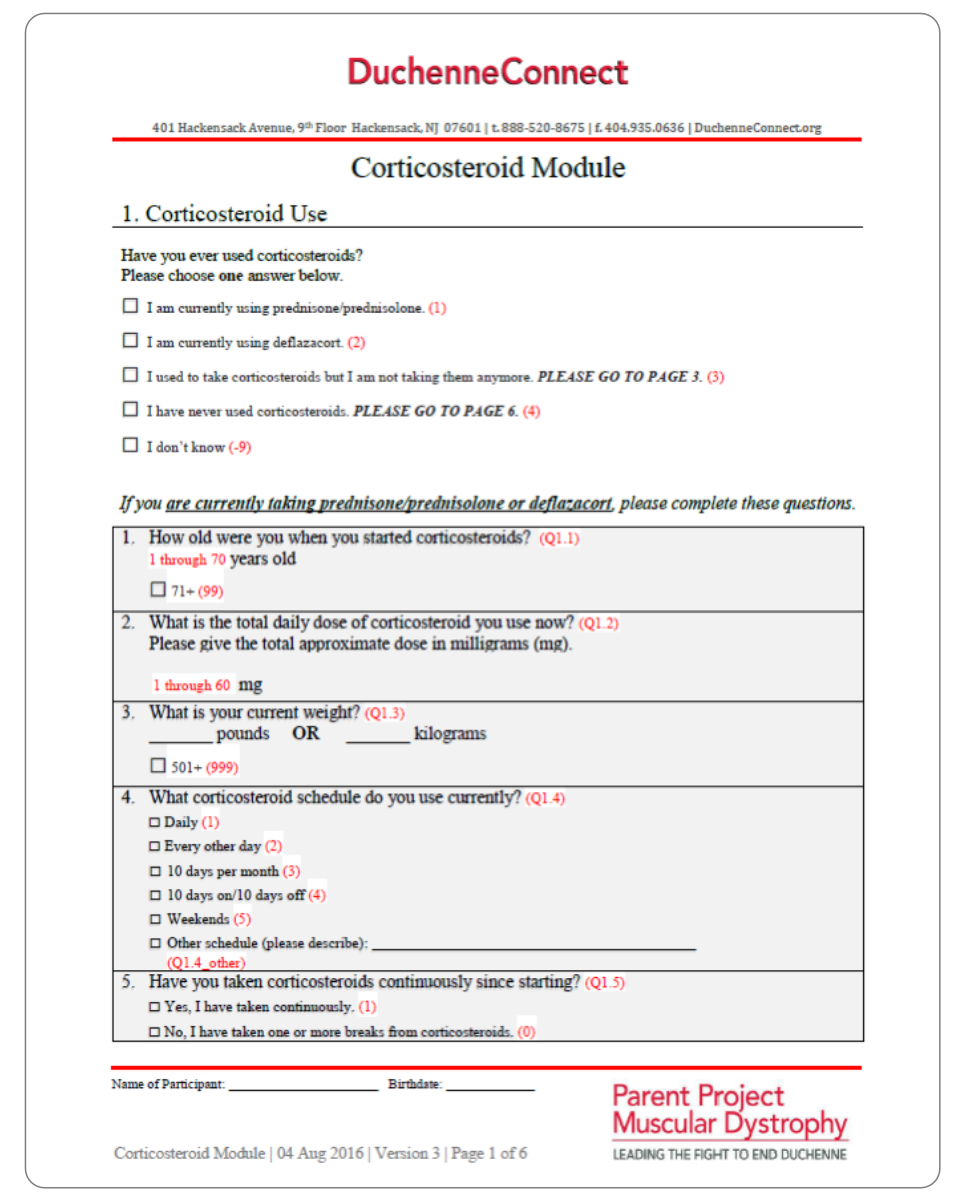
OBJECTIVE

Objective of this study

- To understand the landscape of steroid use in individuals affected by Duchenne and Becker in the U.S.

Analysis of the DuchenneConnect database

- Analysis of responses from individuals in the U.S. under age 35 affected by Duchenne or Becker (all ages)
  - 83% of Duchenne participants were age 18 or less



DuchenneConnect

401 Hackensack Avenue, 9th Floor Hackensack, NJ 07601 | t.888-520-8675 | f.404.935.0636 | DuchenneConnect.org

Corticosteroid Module

1. Corticosteroid Use

Have you ever used corticosteroids?
Please choose **one** answer below.

☐ I am currently using prednisone/prednisolone. (1)
☐ I am currently using deflazacort. (2)
☐ I used to take corticosteroids but I am not taking them anymore. *PLEASE GO TO PAGE 3.* (3)
☐ I have never used corticosteroids. *PLEASE GO TO PAGE 6.* (4)
☐ I don't know (-9)

If you are currently taking prednisone/prednisolone or deflazacort, please complete these questions.

1. How old were you when you started corticosteroids? (Q1.1)
   1 through 70 years old
   ☐ 71+ (99)
2. What is the total daily dose of corticosteroid you use now? (Q1.2)
   Please give the total approximate dose in milligrams (mg).
   1 through 60 mg
3. What is your current weight? (Q1.3)
   ______ pounds OR ______ kilograms
   ☐ 501+ (999)
4. What corticosteroid schedule do you use currently? (Q1.4)
   ☐ Daily (1)
   ☐ Every other day (2)
   ☐ 10 days per month (3)
   ☐ 10 days on/10 days off (4)
   ☐ Weekends (5)
   ☐ Other schedule (please describe): ______________________
   (Q1.4_other)
5. Have you taken corticosteroids continuously since starting? (Q1.5)
   ☐ Yes, I have taken continuously. (1)
   ☐ No, I have taken one or more breaks from corticosteroids. (0)

Name of Participant: ______________ Birthdate: __________

Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

Corticosteroid Module | 04 Aug 2016 | Version 3 | Page 1 of 6

Variability and trends in corticosteroid use by male United States participants with Duchenne muscular dystrophy in the Duchenne Registry

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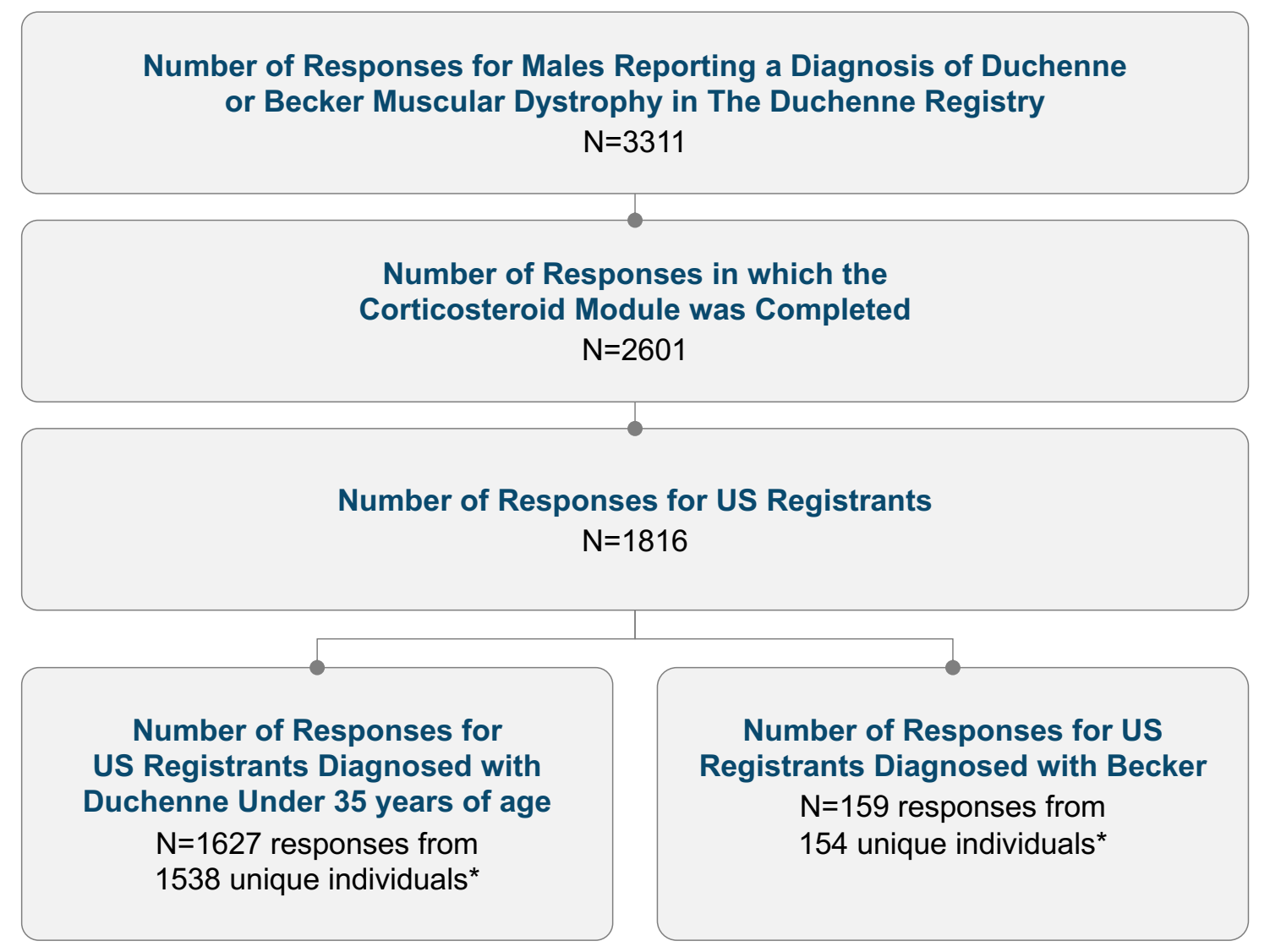
RESULTS

RESPONDENTS



Number of Responses for Males Reporting a Diagnosis of Duchenne or Becker Muscular Dystrophy in The Duchenne Registry
N=3311

Number of Responses in which the Corticosteroid Module was Completed
N=2601

Number of Responses for US Registrants
N=1816

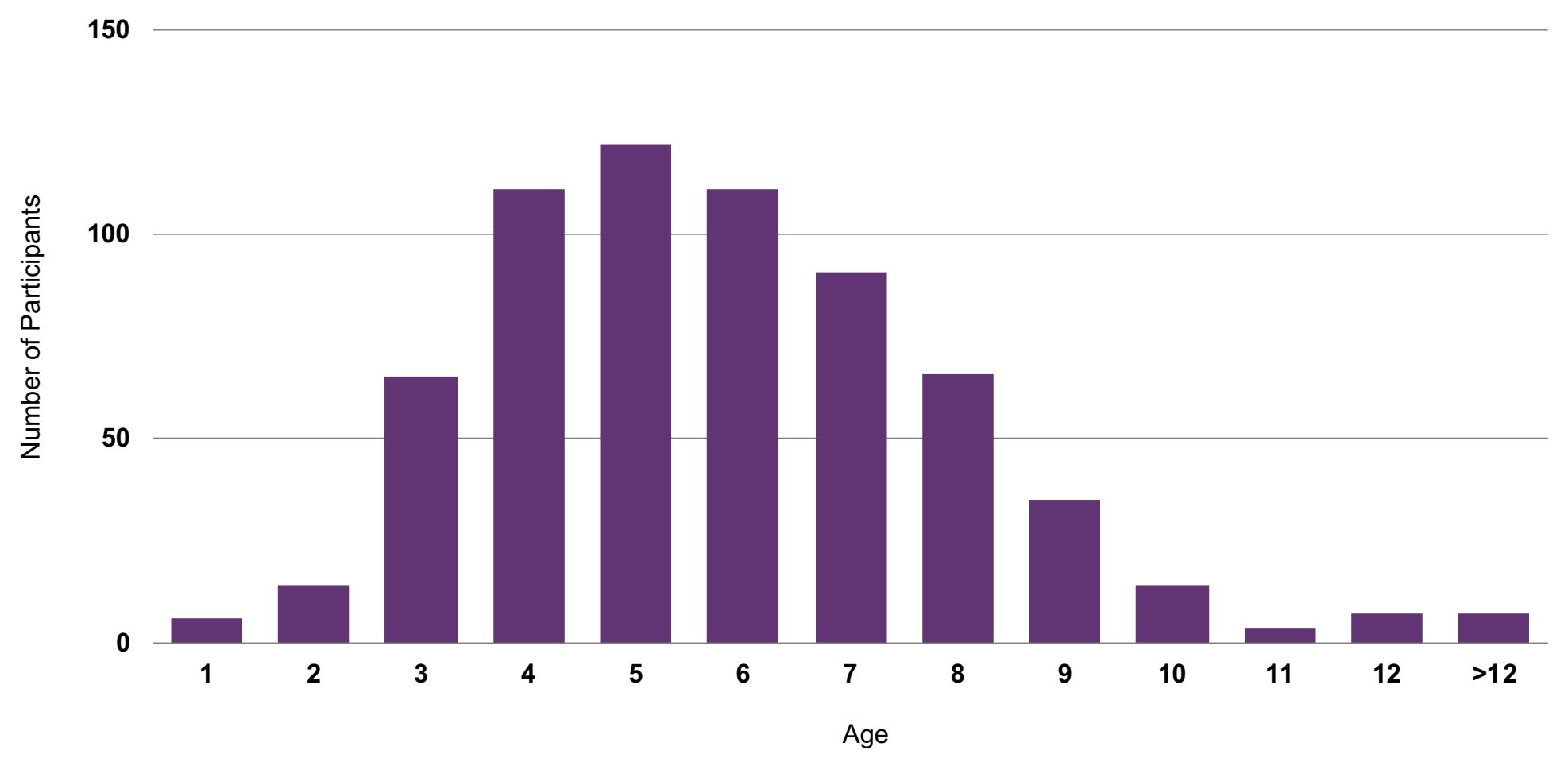
Number of Responses for US Registrants Diagnosed with Duchenne Under 35 years of age
N=1627 responses from 1538 unique individuals*

Number of Responses for US Registrants Diagnosed with Becker
N=159 responses from 154 unique individuals*

* Individuals had multiple responses for some questions in the Corticosteroid Module
Note: Limited data over age 26; prednisolone is included in prednisone counts

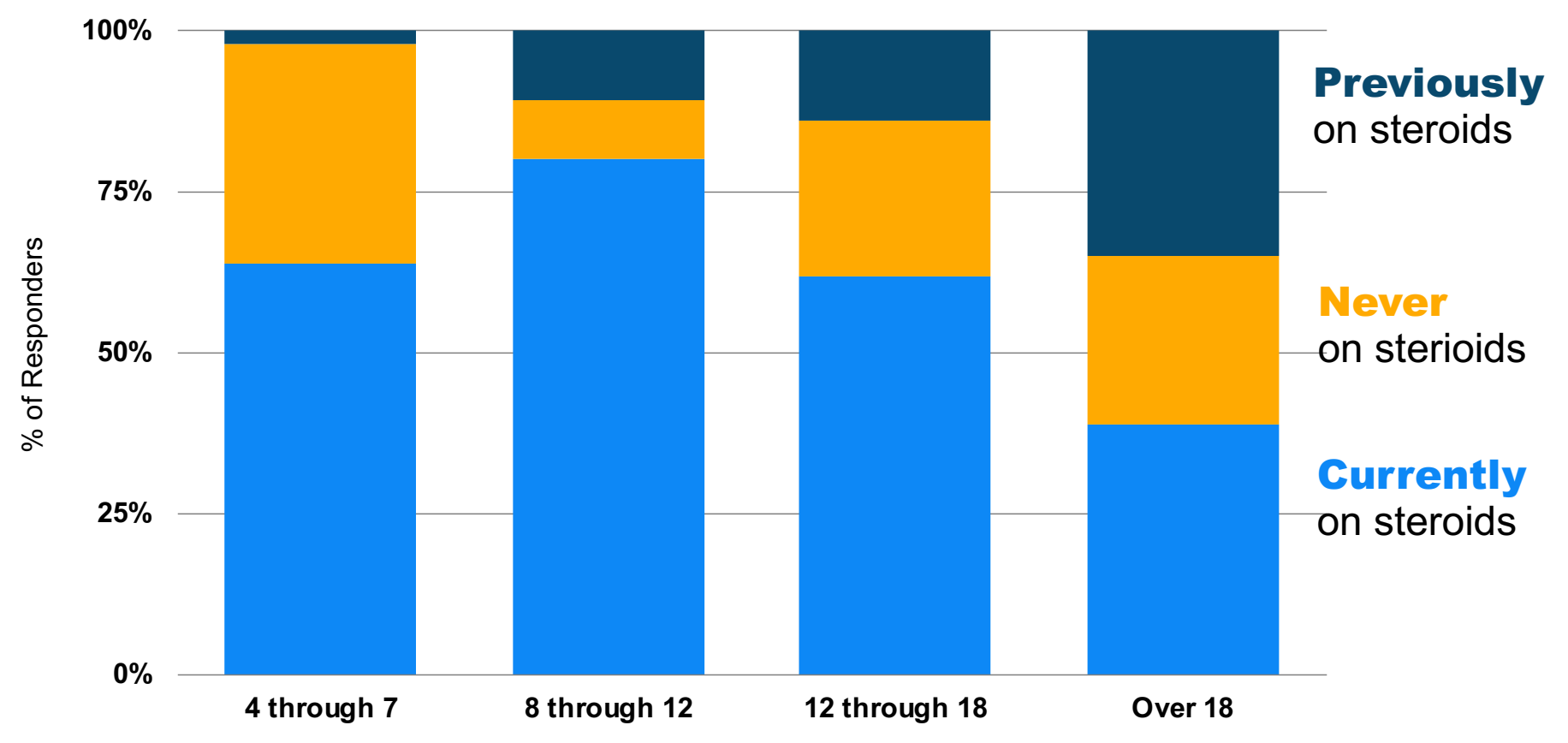
INITIATION OF STEROIDS BY AGE IN DUCHENNE

- Treatment with steroids began on average at age 5.9 years (SD 2.5 years)
- Only 3% of participants reported treatment with steroids began after age 10



STEROID USE BY AGE IN DUCHENNE

- Among all responses from those with Duchenne, 63% were currently on corticosteroids, 12% were no longer on corticosteroids, and 25% had never been on corticosteroids.
- Use of deflazacort (54%) was reported more commonly than prednisone (46%).



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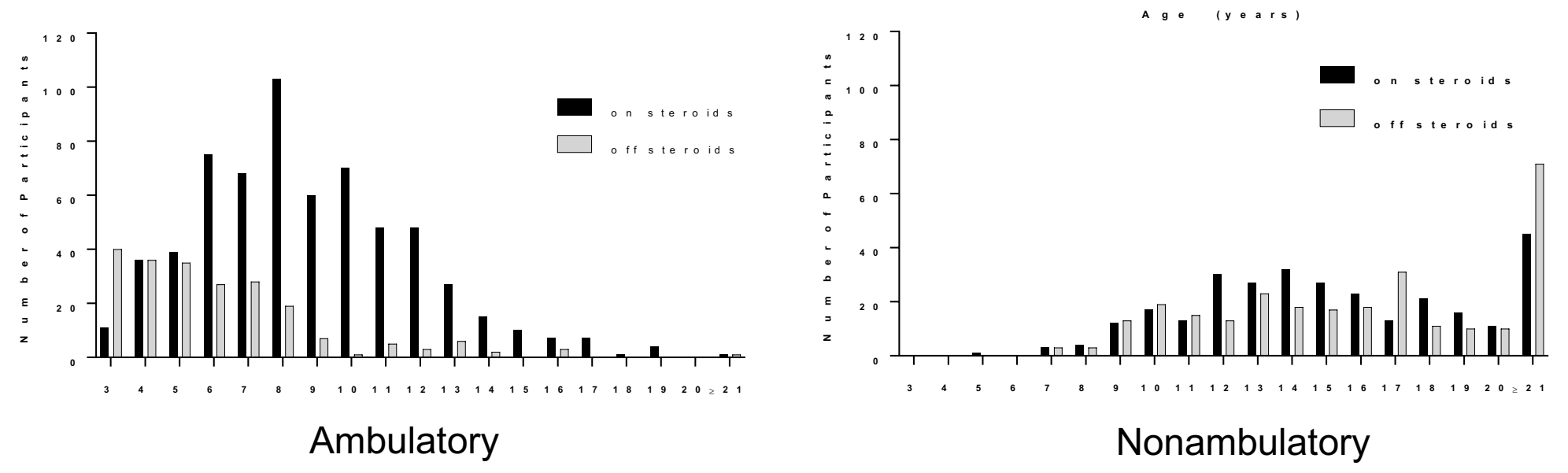
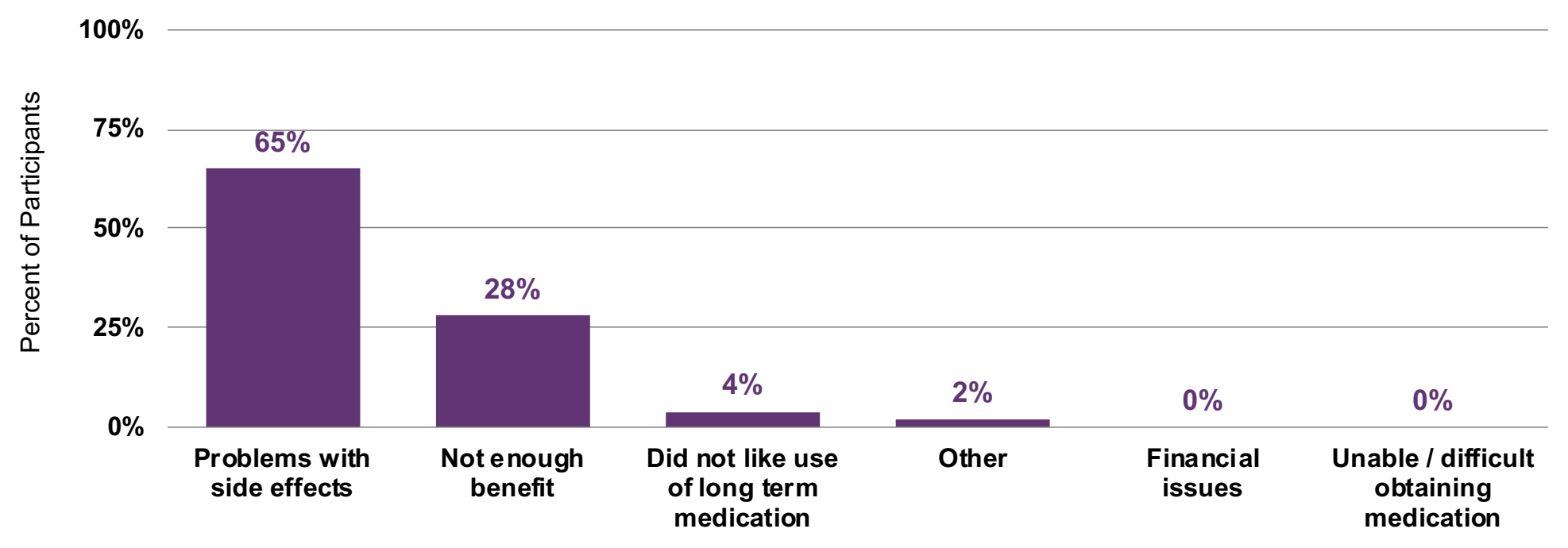
STEROIDS USE BY AGE AND AMBULATORY STATUS

- Among those who were nonambulatory, 49% were currently on corticosteroids, 28% had discontinued corticosteroids, and 23% had never used corticosteroids.
- For the 154 registrants with Becker muscular dystrophy who responded to the Corticosteroid Survey, only 20% were currently using corticosteroids.



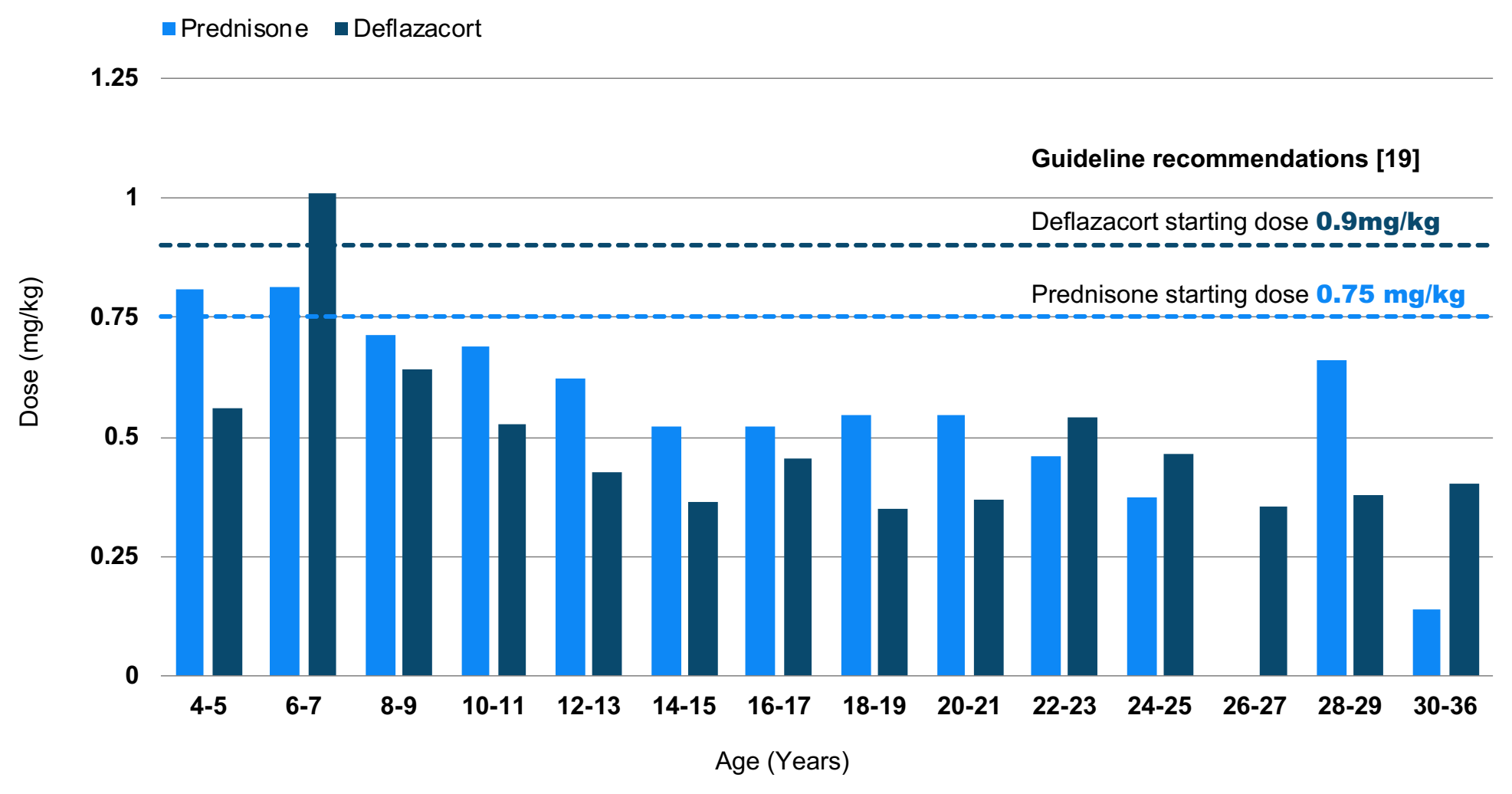
REASONS FOR DISCONTINUING STEROIDS

- Among the options given, the most important reasons for discontinuation of steroids were "problems with side effects" (65%) and "not enough benefit" (28%).
- Secondary reasons for discontinuing steroids mimicked primary reasons: "not enough benefit" (39%) and "problems with side effects" (35%).
  - About half of those participants listing side effects as the secondary reason for discontinuing steroids also listed side effects (or a particular side effect) as the primary reason for discontinuing steroids.



STEROID DOSE BY AGE

Average doses of corticosteroids were below the recommended doses set forth in the Duchenne Care Guidelines (67% of the recommended doses).



Note: Prednisolone is included in prednisone counts

STEROID USE IN DIFFERENT BIRTH INTERVALS

Over the past 5 years compared to the previous 5 years, use of steroids has generally become more common.

- Steroid use in participants over age 18 increased markedly from 24% to 55%.

Birth Interval (years)	Age at initiation of steroids (years, median, 1st and 3rd quartiles)*	N	On steroids %, n		Discontinued %, n		Never on steroids %, n		Age 4 to 7 on steroids**	
1980-89	9 (7, 17)	87	20.7%	18	31.0%	27	48.3%	42	NA	NA
1990-94	8 (6, 10)	138	42.8%	59	29.7%	41	27.5%	38	NA	NA
1995-99	7 (6, 8)	316	66.5%	210	21.2%	67	12.3%	39	NA	NA
2000-04	6 (4, 8)	449	77.7%	349	10.5%	47	11.8%	53	67.6%	46/68
2005-09***	5 (4, 6)	418	75.6%	316	3.3%	14	21.1%	88	69.6%	156/224

* For those who provided age at initiation (overall 60.2%, 692/1148).
** Only available for cohorts in which there were registrants in this age range (i.e., after birth year 2000).
*** Youngest members of this cohort were 7 years of age.

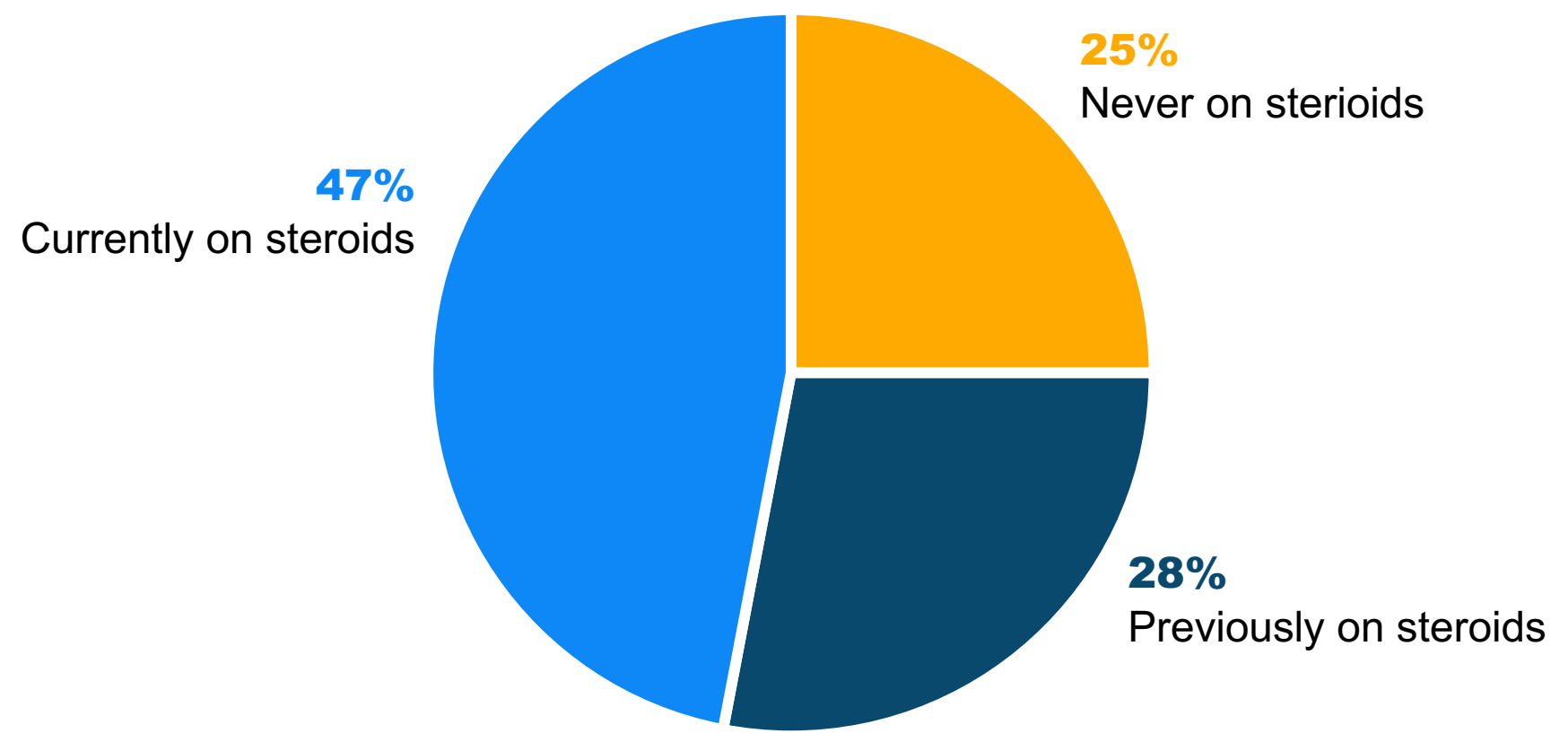
NA = not available

Note: Limited data under age 4 and over age 18

STEROID USE IN NON-AMBULATORY PARTICIPANTS AFFECTED BY DUCHENNE

Steroid use in participants who "rarely or never walk":

- 47% were currently on steroids
- 28% were previously on steroids
- 25% had never been on steroids



CONCLUSIONS & ACKNOWLEDGEMENTS

CONCLUSIONS

Participation in Duchenne registries is valuable to better understand the reasons why individuals and families make treatment decisions.

- Both efficacy and side effects appear to be significant considerations in decision making.

Data provided to The Duchenne Registry furthers the community's understanding of the real-world treatment of Duchenne.

- Despite consensus recommendations, a considerable fraction of participants registered with The Duchenne Registry were not on steroids or not on recommended doses.

Analysis of The Duchenne Registry has expanded our knowledge of steroid use in the U.S.

- These data reinforce the need for additional treatment options for individuals affected by Duchenne.

THANK YOU

- Participants and families
- Parent Project Muscular Dystrophy
- The Duchenne Registry
- Catabasis team

THE DUCHENNE REGISTRY

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