Prosensa Webinar for Patient Groups

Leiden, January 21, 2014

Giles Campion, Chief Medical Officer

Forward-Looking Statements

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Introduction

Giles Campion –
Chief Medical Officer and
SVP R&D

What Will We Discuss?

- Agreement between GSK and Prosensa on transfer of rights
- Results from further analyses of the drisapersen data
- The potential for re-dosing boys
- Next steps
- Q&A session – e-mail questions to patientinfo@prosensa.nl

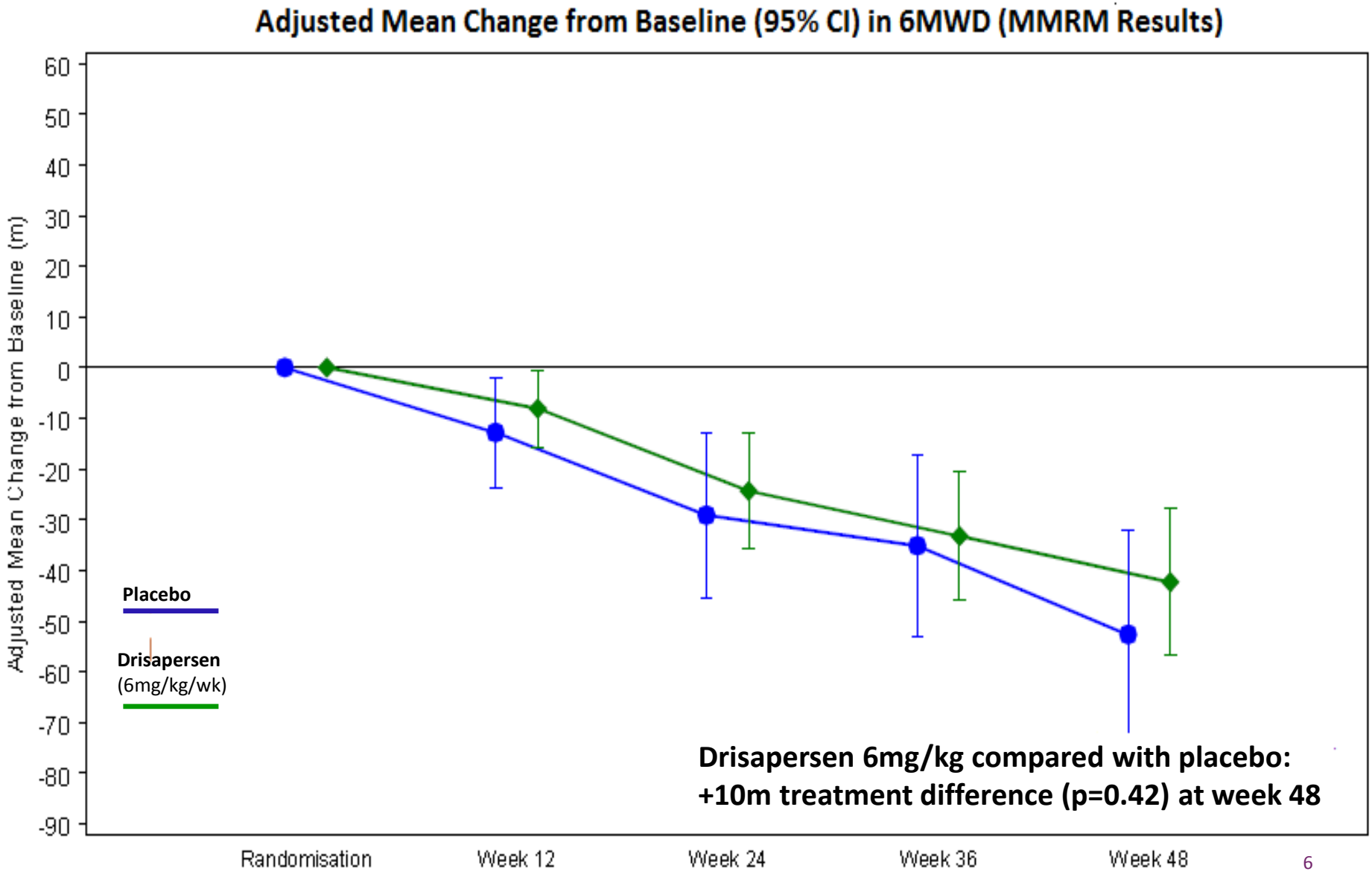
Prosensa Now Responsible

- Prosensa is ‘well suited’ to continue the development of drisapersen
- Prosensa will discuss with key stakeholders potential path forward
  - Patient groups
  - Clinical experts
  - Regulators
- Prosensa will assess feasibility and timing of possible re-dosing

Results of Drisapersen Analyses

- Since September 20, 2013, GSK and Prosensa initiated analyses
  - Phase III study, in context of other studies
  - Integrated analyses
  - Long term open label studies
  - Subsets of boys
- Robust process, maintaining integrity of the database
- Support from patient groups, parents, experts, colleagues
- Work not yet complete, but preliminary conclusions emerge

Phase III Study - Results



Phase III Study - Conclusions

- Primary endpoint week 48: no statistically significant or clinically meaningful treatment difference (10.3m; p=0.42) over placebo in 6MWD
- No statistically significant or clinically meaningful treatment differences between drisapersen and placebo on the majority of secondary endpoints
- A statistically significant (p<0.001) decline in CK (creatine kinase), a potential marker of muscle cell damage, was observed at week 48 for the drisapersen group compared with placebo
- Pre planned subgroup <= 7 years showed a non statistically significant treatment difference of 21m

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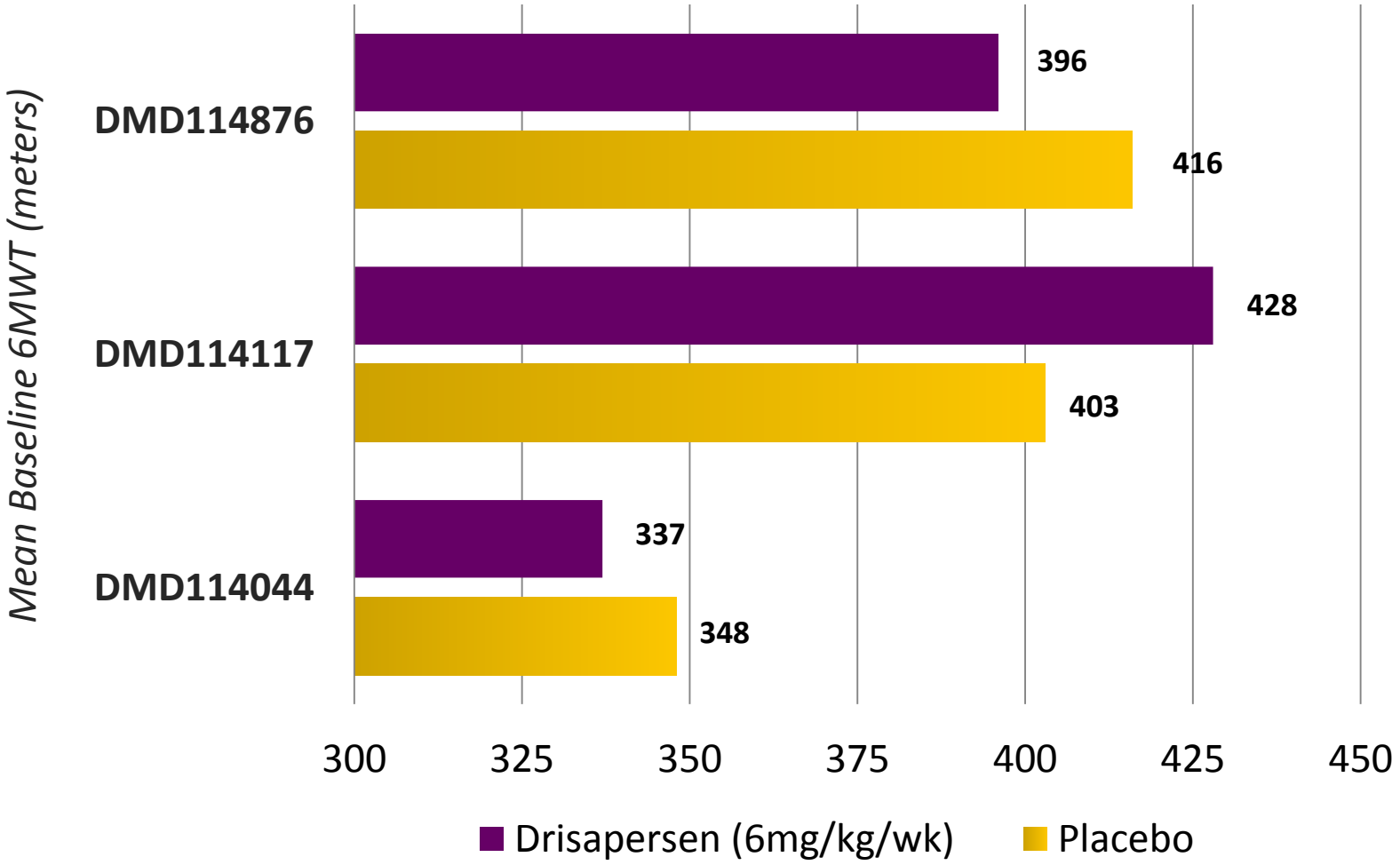
- Boys in the phase III study were generally more advanced in their disease than in previous studies

Comparison of Boys Entering the Study

Study	Treatment drisapersen 6mg/kg/wk	Time since first symptoms (months), mean (sd)	Time since diagnosis (months), mean (sd)	Age (yrs), mean (sd), range
DMD114876	Placebo	57 (30)	46 (30)	8.0 (1.8) 5-11
	Drisapersen	59 (29)	47 (27)	7.6 (2.7) 5-13
DMD114117	Placebo	64 (24)	44 (22)	6.9 (1.2) 5-9
	Drisapersen	61 (25)	45 (28)	7.2 (1.7) 5-11
DMD114044	Placebo	67 (31)	54 (33)	8.0 (2.4) 5-16
	Drisapersen	72 (31)	58 (35)	8.3 (2.4) 5-16

Comparison of Mean Baseline 6MWD



Pooled Results - Preliminary Conclusions

- Pooling the results of the phase III study (DMD114044) with previous placebo controlled studies (DMD114117 and DMD114876)
  - No clinically meaningful difference, possibly due to large impact of phase III study

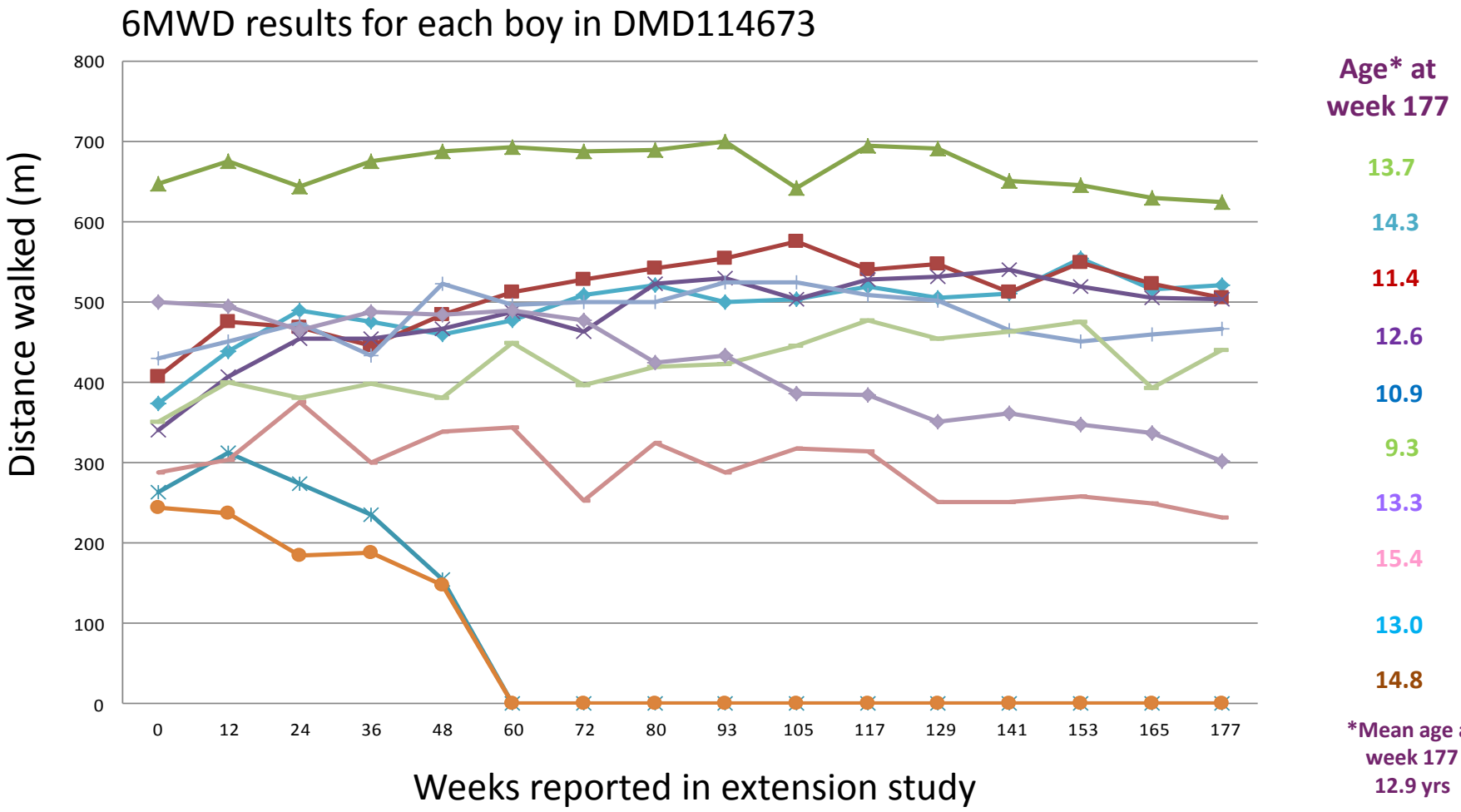
- Integrated analysis of two placebo controlled studies with similar boys enrolled
  - DMD114117 (DEMAND II) and DMD114876 (DEMAND V)
  - Clinically meaningful and statistically significant outcome

Long Term Open Label Trials

- DMD114673 - Extension study after initial dose finding study
  - 12 boys treated for > 4 years, results of 177 weeks

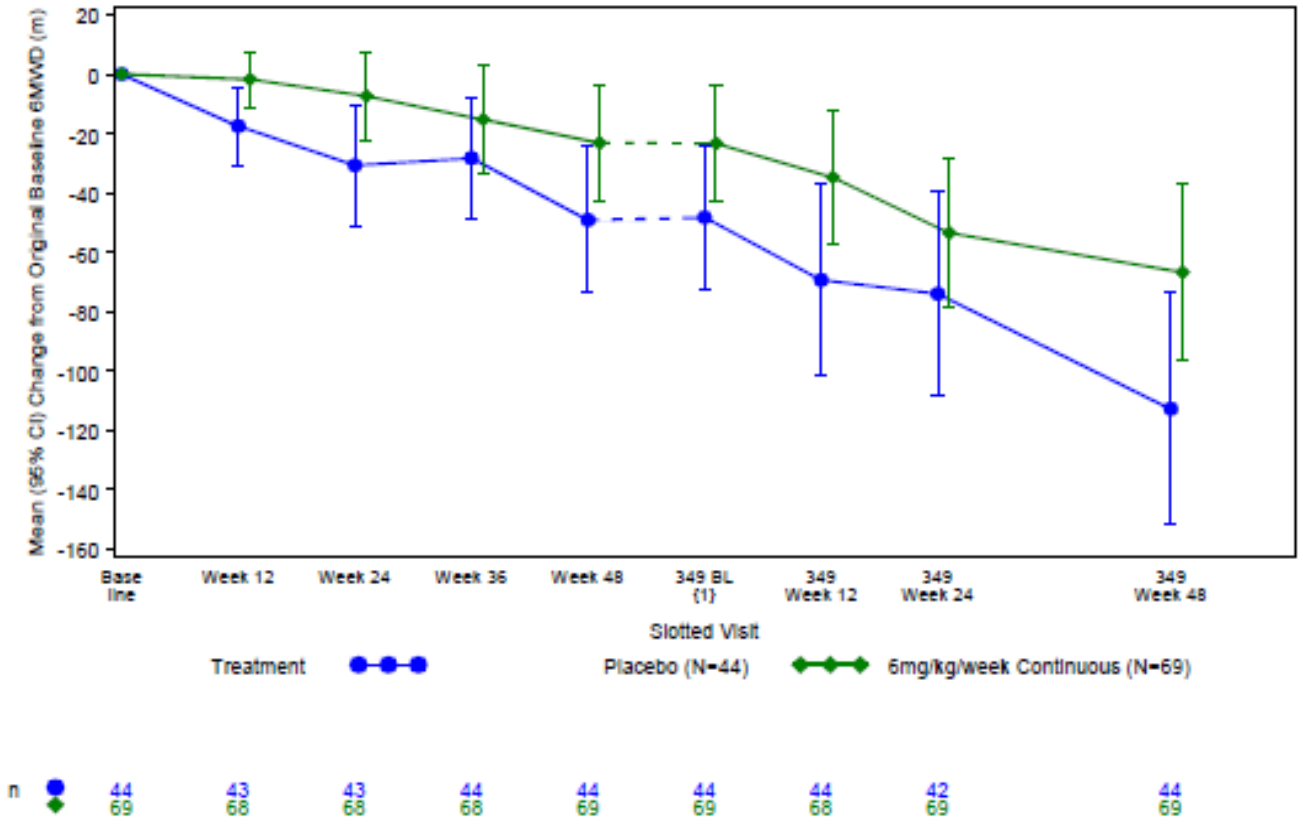
- DMD114349 - Extension study of DMD114044 and DMD114117 study
  - 96 weeks results available

DMD114673 - Results after 177 Weeks



DMD114349 - Results after 96 Weeks

96 weeks from studies DMD114117 and DMD114044



Mean changes from original baseline at DMD114349 week 48:

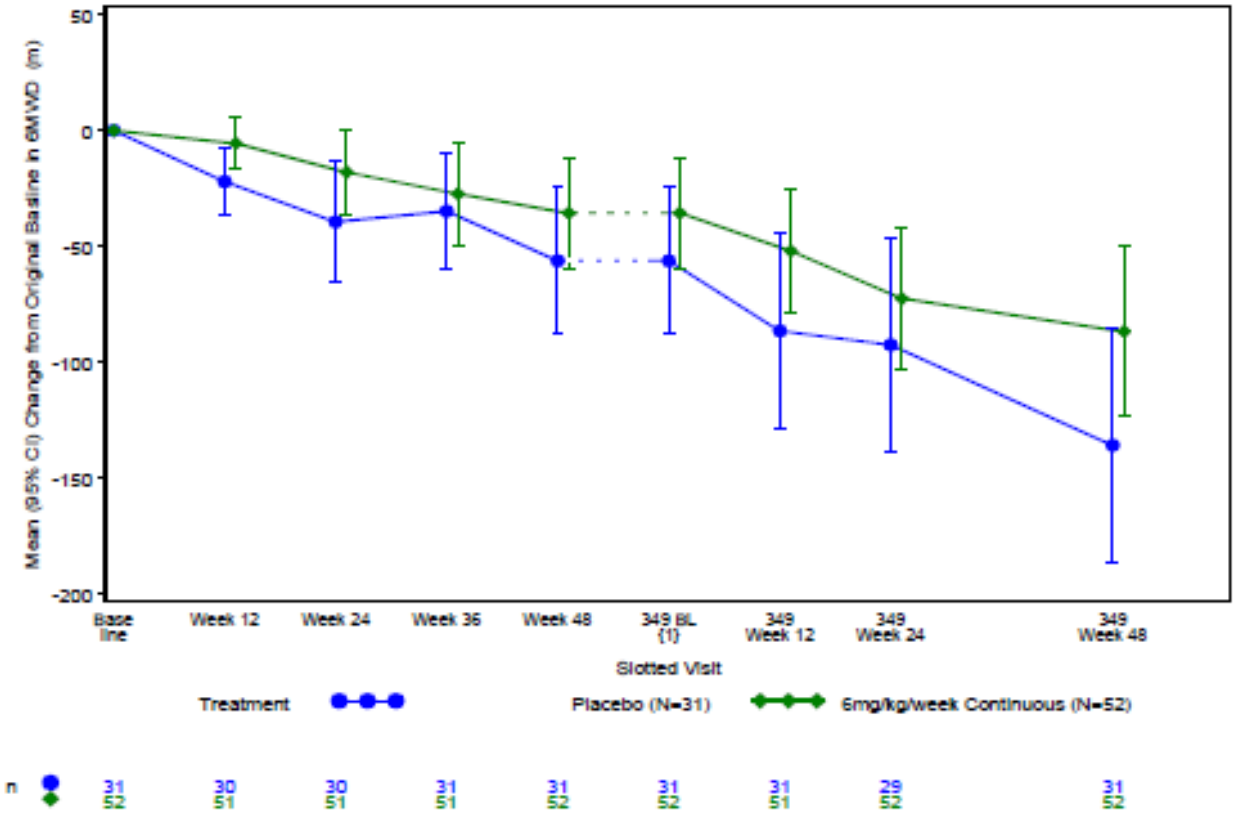
Placebo/Delayed:
-113m (n=44)
Continuous:
-67m (n=69)

Treatment difference:
+46m (n=113)

Summary includes Subjects Reaching Week 48 in Study DMD114349, by Original Treatment Group – ITT Population, Subjects Receiving Either Regimen in DMD114349

DMD114349 - Results after 96 Weeks

96 weeks from study DMD114044



Mean changes from original baseline at DMD114349 week 48:

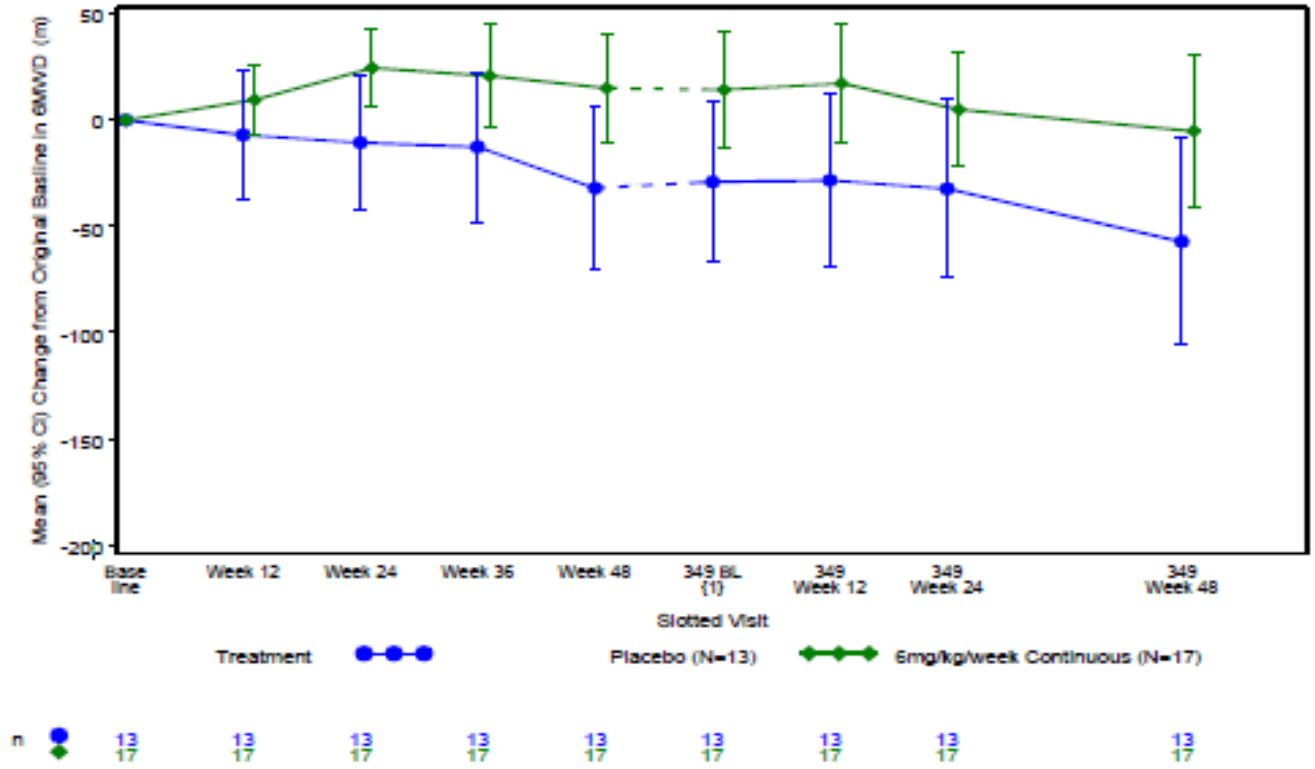
Placebo/Delayed:
-136m (n=31)
Drisapersen:
-87m (n=52)

Treatment difference:
+49m (n=83)

Summary includes Subjects Reaching Week 48 in Study DMD114349, by Original Treatment Group – ITT Population, Subjects Receiving Either Regimen in DMD114349

DMD114349 - Results after 96 Weeks

96 weeks from study DMD114117



Mean changes from original baseline at DMD114349 week 48:

Placebo/Delayed:
-57m (n=13)
Drisapersen:
-5m (n=17)

Treatment difference:
+52m (n=30)

Summary includes Subjects Reaching Week 48 in Study DMD114349, by Original Treatment Group – ITT Population, Subjects Receiving Either Regimen in DMD114349

Subset - Age ≤ 7 Years

Study	n	Week	Treatment difference	P-value
DMD114876	16	24	+31m	0.291
DMD114117	24	49	+38m	0.134
DMD114044	79	48	+21m	0.131
DMD114117 & DMD114044	103	48	+24m	0.048
DMD114349	52	96	+37m	NA

Subset - Age > 7 Years

Study	n	Week	Treatment difference	P-value
DMD114876	18	24	+28m	0.131
DMD114117	11	49	+56m	0.086
DMD114044	107	48	+7m	0.703
DMD114117 & DMD114044	109	48	+7m	0.670
DMD114349	61	96	+62m	NA

Key Safety Data

- Most relevant adverse events include

- Injection site reactions,
- Renal adverse events, including subclinical proteinuria, and
- Thrombocytopenia

- Some of these events have been moderate to severe

- For more information on safety findings: www.gsk-clinicalstudyregister.com

Preliminary Efficacy Conclusions

- The data suggest that boys who are less progressed in their disease show a treatment difference on the six-minute walk test by 24 or 48 weeks
- The data suggest that longer treatment may be needed to show a treatment difference in more progressed boys
- These findings are preliminary and will be discussed with DMD experts

Sharing of Results

- Presentation in scientific meetings
- Publications in peer reviewed medical journals
- Relevant placebo data in drisapersen studies with scientific community
- Publication of dystrophin methodology and sharing with ‘biomarker-group’

Natural History Study

- Observational study
- Up to 250 boys to be enrolled, over 150 boys participate so far
- Information on www.clinicaltrials.gov
- The study aims to:
  - characterize the natural history and progression of DMD
  - help inform the design of future studies
  - capture biomarkers of safety and disease progression
  - provide comparative data for the development of rare exons for which formal controlled trials are not feasible.

Re-dosing Possibilities

- Completion of analyses of drisapersen results across all studies
- Engagement with patient groups, clinical experts and regulators
- Possibilities for re-initiation of dosing will be assessed based on feedback from these stakeholders
- Until further notice, dosing of patients on hold; scheduled visits, safety monitoring and relevant assessments continue

- We realize all to well that this is a very difficult period for the boys and families who are waiting for a final decision and we will work as swiftly as possible

Impact on Other DMD Programs

- Ongoing studies continue
- Learning from drisapersen study results
- Based on outcome of full evaluation drisapersen, possible adaptation
- Currently too early to speculate about the impact on other compounds
- As soon as we have more clarity, we will provide additional information

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- Prosensa is committed to finding a solution for as many DMD boys as possible

Committed to DMD

- We realize that the road towards access to a new orphan medicinal product for patients is often very difficult
- Given the unmet medical need and the severity of this disease, we feel obliged to explore all possibilities in depth and to leave no stone unturned
- We remain confident in our exon skipping technology are fully committed to bringing a treatment to Duchenne patients
- We will keep you informed

Thank You!

- We would like to thank all the boys that have participated or are participating in the Prosensa clinical programs
- We realize how much commitment and time to participate in a clinical study and how difficult this period is
- We would also like to thank the community at large for their input, confidence and patience

- If you would like to discuss the implications for your son, we recommend you to contact the treating physician

- Investigators have had separate briefings of this information

- Questions to Prosensa: patientinfo@prosensa.nl

PROSENSA

RNA modulation to fight Duchenne Muscular Dystrophy