UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): May 3, 2018

Sarepta Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**001-14895**	**93-0797222**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

215 First Street
Suite 415
Cambridge, MA 02142
(Address of principal executive offices, including zip code)

(617) 274-4000
(Registrant's telephone number, including area code)

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01. Regulation FD Disclosure.

On May 3, 2018, Sarepta Therapeutics, Inc. issued a press release announcing, among other things, that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has rendered a negative trend vote following an oral explanation of the Company's Marketing Authorization Application (MAA) for eteplirsen. The Company plans to seek a re-examination and request that a Scientific Advisory Group (SAG) be convened to provide expert guidance and insight into different neuromuscular issues, including the validity of the external controls used and the importance of significantly slowing pulmonary decline in patients with Duchenne muscular dystrophy. A copy of the press release is furnished as Exhibit 99.1 and is incorporated herein by reference, including the cautionary statement on forward-looking statements included in the press release regarding the Company's plan to seek a re-examination and request that a SAG be convened.

At the oral explanation, the Company presented and had a Q&A session with CHMP members per standard meeting protocol. A copy of the presentation is furnished as Exhibit 99.2 and is incorporated herein by reference.

The information in this report furnished pursuant to Item 7.01, including Exhibits 99.1 and 99.2 attached hereto, shall not be deemed "filed" for the purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section. It may only be incorporated by reference in another filing under the Exchange Act or the Securities Act of 1933, as amended, if such subsequent filing specifically references the information furnished pursuant to Item 7.01 of this report.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit Number	Description
99.1	Press release dated May 3, 2018.
99.2	Oral Explanation Presentation Slides

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Sarepta Therapeutics, Inc.

By: /s/ Douglas S. Ingram
Douglas S. Ingram
President and Chief Executive Officer

Date: May 3, 2018

Exhibit 99.1

Sarepta Therapeutics Announces First Quarter 2018 Financial Results and Recent Corporate Developments

— First quarter 2017 EXONDYS 51® (eteplirsen) total net revenues of $64.6 million —

— Sarepta signs exclusive partnership and buy-out option with Myonexus Therapeutics; pipeline expands from 16 to 21 programs —

— Company announces date of first R&D day, at which clinical data from gene therapy micro-dystrophin program will be announced —

— Company receives negative trend vote following its CHMP oral explanation; will request re-examination and Scientific Advisory Group to be convened —

CAMBRIDGE, Mass., May 3, 2018 (GLOBE NEWSWIRE) — Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases, today reported financial results for the three months ended March 31, 2018.

“In the first quarter, we continued our successful launch of EXONDYS 51 and advanced our pipeline to bring life-enhancing therapies to those suffering from rare disease around the world,” said Doug Ingram, Sarepta’s president and chief executive officer. “We accelerated our gene therapy and RNA platform, and in that regard are excited to announce that our first R&D day will take place on June 19 to showcase the breadth, depth and progress of our pipeline. Significantly, at this event we will report preliminary safety and gene expression data from at least two patients from our micro-dystrophin gene therapy trial underway with Nationwide Children’s Hospital.”

Mr. Ingram continued, “Aligned with our stated goal of leveraging our expertise beyond DMD, we announced today a collaboration with Myonexus Therapeutics for the development of five potentially transformative gene therapies to treat a debilitating set of diseases, all under the umbrella of Limb-girdle muscular

dystrophy. Through this collaboration, we have expanded our pipeline to 21 therapies in development. Our confidence in the Myonexus collaboration comes from the similarities between the Myonexus and Sarepta approaches to gene therapy. Both are seeking to treat rare neuromuscular disease through the AAVrh.74 vector; and both rely upon the unparalleled expertise of Dr. Louise Rodino-Klapac in developing and executing gene therapy constructs. This partnership with Myonexus enables us to expand our efforts beyond DMD while maintaining our unwavering commitment to those suffering from DMD."

Mr. Ingram concluded, "Unfortunately, in addition to our successes in the first quarter, we also have had a delay in our effort to bring eteplirsen to patients in Europe who could potentially benefit from it. I could not be prouder of our Sarepta team and the team of experts who spoke on behalf of eteplirsen at the CHMP oral explanation last week. The rigorous work that was done to prepare for the hearing only strengthened our resolve that eteplirsen should urgently be made available to those waiting in Europe. Unfortunately, the CHMP's trend vote was negative. Based on discussions with CHMP representatives, it is our understanding that the CHMP did not conclude that eteplirsen is ineffective for exon 51 amenable patients, but rather that Sarepta has not yet met the regulatory threshold for conditional approval, in part due to the use of external controls as comparators in the studies. Sarepta plans to file for re-examination and will request that a Scientific Advisory Group (SAG), which is made up of DMD and neuromuscular specialists, be convened to provide expert guidance and insight into, among other things, the validity of the external controls used and the importance of slowing pulmonary decline in patients with DMD."

Financial Results

For the first quarter of 2018, on a GAAP basis, Sarepta reported a net loss of $35.4 million, or $0.55 per basic and diluted share, compared to net income of $84.1 million reported for the same period of 2017, or $1.53 per basic share and $1.50 per diluted share. On a non-GAAP basis, the net loss for the first quarter of 2018 was $17.9 million, or $0.28 per share, compared to a net loss of $31.4 million for the same period of 2017, or $0.57 per share.

Net Revenues

For the three months ended March 31, 2018, the Company recorded net product revenues of $64.6 million, compared to net revenues of $16.3 million for first quarter of 2017. The increase primarily reflects increasing demand for EXONDYS 51 in the U.S.

Cost and Operating Expenses

Cost of sales (excluding amortization of in-licensed rights)

For the three months ended March 31, 2018, cost of sales (excluding amortization of in-licensed rights) was $5.6 million, compared to $0.2 million for the same period of 2017. The increase primarily reflects royalty payments to BioMarin Pharmaceuticals (BioMarin) as a result of the execution of the settlement and license agreements with BioMarin in July 2017 as well as higher inventory costs related to increasing demand for EXONDYS 51 during 2018. Prior to the approval of EXONDYS 51, the Company expensed related manufacturing and material costs as research and development expenses.

Research and development

Research and development expenses were $46.2 million for the first quarter of 2018, compared to $29.1 million for the same period of 2017, an increase of $17.1 million. The increase in research and development expenses primarily reflects the following:

- $4.4 million increase in clinical and manufacturing expenses primarily due to increased patient enrollment in the Company's ongoing clinical trials in golodirsen and casimersen, as well as a ramp-up of manufacturing activities for the Company's PPMO platform. These increases were partially offset by a ramp-down of clinical trials in eteplirsen primarily because the PROMOVI trial has been fully enrolled;
- $3.2 million increase in collaboration cost sharing with Summit on its utrophin platform;
- $2.7 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $2.4 million increase in professional services primarily due to an expansion of the Company's research and development pipeline; and
- $1.6 million increase in preclinical expenses primarily due to the continuing ramp-up of toxicology studies in the Company's PPMO platform as well as golodirsen and casimersen.

Non-GAAP research and development expenses were $43.3 million for the first quarter of 2018, compared to $26.7 million for the same period of 2017, an increase of $16.6 million.

Selling, general and administration

Selling general and administrative expenses were $43.3 million for the first quarter of 2018, compared to $26.2 million for the same period of 2017, an increase of $17.1 million. The increase in selling, general and administrative expenses primarily reflects the following:

- $6.4 million increase in professional services primarily due to continuing global expansion as well as preparation for a potential product launch in the EU should the Company's Marketing Authorization Application be approved by the European Medicines Agency;
- $5.3 million increase in compensation and other personnel expenses primarily due to a net increase in headcount; and
- $4.6 million increase in stock-based compensation primarily due to the impact of revising the forfeiture rate assumption for officers and Board of Directors as well as an increase in stock price.

Non-GAAP selling, general and administrative expenses were $33.7 million for the first quarter of 2018, compared to $21.1 million for the same period of 2017, an increase of $12.6 million.

Amortization of in-licensed rights

Amortization of in-licensed rights was $0.2 million during the first quarter of 2018, compared to less than $0.1 million for the same period of 2017. The increase was primarily due to the BioMarin transactions that occurred in July 2017.

Other (loss) Income

Gain from sale of Priority Review Voucher

In connection with the completion of the sale of the Priority Review Voucher (PRV) in March 2017, the Company recorded a gain of $125.0 from sale of the PRV in the first quarter of 2017. There was no similar activity in the first quarter of 2018.

Interest (expense) income and other, net

For the three months ended March 31, 2018 and 2017, the Company recorded $4.5 million interest expense and other, net and $0.3 interest income and other, net, respectively. The period over period unfavorable change primarily reflects the interest expense accrued on the Company's debt facilities partially offset by interest income from higher balances of cash, cash equivalents and investments.

Cash, Cash Equivalents, Restricted Cash and Investments

The Company had $1.0 billion in cash, cash equivalents, restricted cash and investments as of March 31, 2018 compared to $1.1 billion as of December 31, 2017. The decrease is primarily driven by the use of cash to fund the Company's ongoing operations during the first quarter of 2018.

Use of Non-GAAP Measures

In addition to the GAAP financial measures set forth in this press release, the Company has included certain non-GAAP measurements. The non-GAAP loss is defined by the Company as GAAP net loss excluding interest expense/(income), income tax expense/(benefit), depreciation and amortization expense, stock-based compensation expense, restructuring expense and other items. Non-GAAP research and development expenses are defined by the Company as GAAP research and development expenses excluding depreciation and amortization expense, stock-based compensation expense, restructuring expense and other items. Non-GAAP selling, general and administrative expenses are defined by the Company as GAAP selling, general and administrative expenses excluding depreciation and amortization expense, stock-based compensation expense, restructuring expense and other items.

1. Interest, tax, depreciation and amortization

Interest income and expense amounts can vary substantially from period to period due to changes in cash and debt balances and interest rates driven by market conditions outside of the Company's operations. Tax amounts can vary substantially from period to period due to tax adjustments that are not directly related to underlying operating performance. Depreciation expense can vary substantially from period to period as the purchases of property and equipment may vary significantly from period to period and without any direct correlation to the Company's operating performance. Amortization expense associated with in-licensed rights as well as patent costs are amortized over a period of several years after acquisition or patent application or renewal and generally cannot be changed or influenced by management.

2. Stock-based compensation expenses

Stock-based compensation expenses represent non-cash charges related to equity awards granted by Sarepta. Although these are recurring charges to operations, management believes the measurement of these amounts can vary substantially from period to period and depend significantly on factors that are not a direct consequence of operating performance that is within management's control. Therefore, management believes that excluding these charges facilitates comparisons of the Company's operational performance in different periods.

3. Restructuring expenses

The Company believes that adjusting for these items more closely represents the Company's ongoing operating performance and financial results.

4. Other items

The Company evaluates other items of expense and income on an individual basis. It takes into consideration quantitative and qualitative characteristics of each item, including (a) nature, (b) whether the items relates to the Company's ongoing business operations, and (c) whether the Company expects the items to continue on a regular basis. These other items include the aforementioned gain from the sale of the Company's PRV.

The Company uses these non-GAAP measures as key performance measures for the purpose of evaluating operational performance and cash requirements internally. The Company also believes these non-GAAP measures increase comparability of period-to-period results and are useful to investors as they provide a similar basis for evaluating the Company's performance as is applied by management. These non-GAAP measures are not intended to be considered in isolation or to replace the presentation of the Company's financial results in accordance with GAAP. Use of the terms non-GAAP research and development expenses, non-GAAP selling, general and administrative expenses, non-GAAP other income adjustments, non-GAAP income tax expense, non-GAAP net loss, and non-GAAP basic and diluted net loss per share may differ from similar measures reported by other companies, which may limit comparability, and are not based on any comprehensive set of accounting rules or principles. All relevant non-GAAP measures are reconciled from their respective GAAP measures in the attached table "Reconciliation of GAAP to Non-GAAP Net Loss."

First Quarter and Recent Corporate Developments

- **Golodirsen (SRP-4053):** Based on Sarepta's Type C meeting with the FDA's Division of Neurology Products to solicit the Division's guidance on the development pathway for golodirsen, the Company remains on track to complete a rolling NDA submission by year-end 2018, seeking accelerated approval based on an increase in dystrophin protein as a surrogate endpoint.
- **Myonexus Therapeutics Partnership:** Sarepta and Myonexus Therapeutics entered into a partnership to advance multiple gene therapies for various forms of Limb-girdle muscular dystrophies (LGMDs). The lead program, MYO-101, has generated encouraging pre-clinical safety and efficacy

data utilizing the AAVrh.74 vector system, the same vector used in the micro-dystrophin gene therapy program Sarepta is developing with Nationwide Children's Hospital. A Phase 1/2a study of MYO-101 is scheduled to begin in mid-2018. The companies plan to report on 60-day biopsy data in late-2018 or early 2019. Additionally, Myonexus is advancing MYO-102 for LGMD2D, MYO-103 for LGMD2C, MYO-201 for LGMD2B, and MYO-301 for LGMD2L. Under the terms of the agreement, Sarepta will make an upfront payment of $60 million and additional development-related milestone payments to purchase an exclusive option to acquire Myonexus at a pre-negotiated, fixed price with sales-related contingent payments. If all development-related milestone payments are met, Sarepta will make payments of up to $45 million over an approximately two-year evaluation period. Sarepta has the option to purchase Myonexus at any time, including upon review of proof-of-concept data.

- **Sarepta R&D Day (Tuesday, June 19, 2018):** Sarepta management, along with several key-opinion leaders, will provide an in-depth look into the Company's pipeline programs across several modalities, included RNA-targeted therapies, gene therapy and gene editing. Of particular note, we look forward to presenting our micro-dystrophin expression data from at least two patients enrolled in the Phase 1/2a gene therapy clinical trial underway with Drs. Jerry Mendell and Louise Rodino-Klapac of Nationwide Children's Hospital. To date, the Company has enrolled four patients in this study and no significant adverse events have been reported. In addition, Dr. Rodino-Klapac, who is also chief scientific officer and co-founder of Myonexus, will present data from Myonexus' entire LGMD program. For all to access, Sarepta's R&D day will be webcast live under the investor relations section of the Company's website at: www.sarepta.com and will be archived there following the event for 90 days.

Conference Call

The Company will be hosting a conference call at 4:30 p.m. Eastern Time, to discuss these financial results and provide a corporate update. The conference call may be accessed by dialing 844-534-7313 for domestic callers and +1-574-990-1451 for international callers. The passcode for the call is 2798939. Please specify to the operator that you would like to join the "Sarepta First Quarter 2018 Earnings Call". The conference call will be webcast live under the investor relations section of Sarepta's website at www.sarepta.com and will be archived there following the call for 90 days. Please connect to Sarepta's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary.

About EXONDYS 51

EXONDYS 51 uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. EXONDYS 51 is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

Important Safety Information About EXONDYS 51

Hypersensitivity reactions, including rash and urticaria, pyrexia, flushing, cough, dyspnea, bronchospasm, and hypotension, have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

Adverse reactions in DMD patients (N=8) treated with EXONDYS 51 30 or 50 mg/kg/week by intravenous (IV) infusion with an incidence of at least 25% more than placebo (N=4) (Study 1, 24 weeks) were (EXONDYS 51, placebo): balance disorder (38%, 0%), vomiting (38%, 0%) and contact dermatitis (25%, 0%). The most common adverse reactions were balance disorder and vomiting. Because of the small numbers of patients, these represent crude frequencies that may not reflect the frequencies observed in practice. The 50 mg/kg once weekly dosing regimen of EXONDYS 51 is not recommended.

In the 88 patients who received ³30 mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in ³10% of patients and occurred more frequently than on the same dose in Study 1: vomiting, contusion, excoriation, arthralgia, rash, catheter site pain, and upper respiratory tract infection.

For further information, please see the full Prescribing Information.

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates. For more information, please visit www.sarepta.com.

Forward-Looking Statements

In order to provide Sarepta's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "may," "intends," "prepares," "looks," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to Sarepta's future operations, financial performance and projections, business plans, priorities and development of product candidates including: Sarepta's goal of leveraging its expertise beyond DMD; expected milestones, including reporting preliminary safety and gene expression data from at least two patients from our micro-dystrophin gene therapy trial on June 19, 2018, completing a rolling NDA submission for golodirsen by year-end 2018, which seeks accelerated approval based on an increase in dystrophin protein as a surrogate endpoint, initiating a Phase 1/2a study of MYO-101 in mid-2018, and reporting on 60-day biopsy data in late-2018 or early 2019; Sarepta's collaboration with Myonexus involving five potentially transformative gene therapies to treat LGMD; the partnership with Myonexus enabling Sarepta to expand its efforts beyond DMD while maintaining its unwavering commitment to those suffering from DMD; payments that Sarepta is expected to make under the agreement with Myonexus; and Sarepta's plan to file for re-examination of its marketing authorization application (MAA) for eteplirsen and to request that a SAG be convened.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: we may not be able to meet expectations with respect to EXONDYS 51 sales or attain the net revenues we anticipate, profitability or positive cash-flow from operations; we may not be able to comply with all FDA post-approval commitments and requirements with respect to EXONDYS 51 in a timely manner or at all; we may not be granted a re-examination of our MAA for eteplirsen, a SAG may not be convened, and even if a re-examination and a related SAG are granted, the CHMP may render a negative opinion and we may not be able to obtain regulatory approval for eteplirsen from the European Medicines Agency; our data for golodirsen may not be sufficient for a filing for or obtaining regulatory approval; the expected benefits and

opportunities related to the agreement with Myonexus may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the partnership with Myonexus may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates or may never become commercialized products due to other various reasons including any potential future inability of the parties to fulfill their commitments and obligations under the agreement, including any inability by us to fulfill our financial commitments to Myonexus; and even if the agreement results in new commercialized products, we may not achieve any significant revenues from the sale of such products; we may not be able to execute on our business plans, including meeting our expectations with respect to EXONDYS 51 sales, meeting our expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing our product candidates to market, for various reasons including possible limitations of Company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, and regulatory, court or agency decisions, such as decisions by the CHMP on eteplirsen or the United States Patent and Trademark Office with respect to patents that cover our product candidates; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2017 and most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company's business, results of operations and the trading price of Sarepta's common stock. You should not place undue reliance on forward-looking statements. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except to the extent required by applicable law or SEC rules.

Internet Posting of Information

We routinely post information that may be important to investors in the 'For Investors' section of our website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

Sarepta Therapeutics, Inc.
Consolidated Statements of Operations
(unaudited, in thousands, except share and per share amounts)

	For the Three Months Ended March 31,	
	2018	2017
Revenues:		
Product, net	$ 64,604	$ 16,342
Total revenues	64,604	16,342
Costs and expenses:		
Cost of sales (excluding amortization of in-licensed rights)	5,582	223
Research and development	46,204	29,119
Selling, general and administrative	43,341	26,216
Amortization of in-licensed rights	216	29
Total costs and expenses	95,343	55,587
Operating loss	(30,739)	(39,245)
Other (loss) income:		
Gain from sale of Priority Review Voucher	—	125,000
Interest (expense) income and other, net	(4,485)	335
Other (loss) income	(4,485)	125,335
(Loss) income before income tax expense	(35,224)	86,090
Income tax expense	139	2,000
Net (loss) income	(35,363)	84,090
Net (loss) income per share		
Basic (loss) earnings per share	$ (0.55)	$ 1.53
Diluted (loss) earnings per share	$ (0.55)	$ 1.50
Weighted average number of shares of common stock used in computing:		
Basic (loss) earnings per share	64,631	54,850
Diluted (loss) earnings per share	64,631	56,012

Sarepta Therapeutics, Inc.
Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures
(unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended March 31,	
	2018	2017
GAAP net (loss) income	$ (35,363)	$ 84,090
Interest expense (income), net	4,503	(29)
Income tax expense	139	2,000
Depreciation and amortization expense	2,252	1,637
Stock-based compensation expense	10,526	5,712
Restructuring expense	—	236
Gain from sale of Priority Review Voucher	—	(125,000)
Non-GAAP net loss (1)	$ (17,943)	$ (31,354)
Non GAAP net loss per share:		
Basic and diluted	$ (0.28)	$ (0.57)
Weighted average number of shares of common stock outstanding for computing:		
Basic and diluted	64,631	54,850

	Three Months Ended March 31,	
	2018	2017
GAAP research and development expenses	46,204	29,119
Stock-based compensation expense	(2,060)	(1,874)
Depreciation and amortization expense	(848)	(512)
Restructuring expense	—	(70)
Non-GAAP research and development expenses (1)	43,296	26,663

	Three Months Ended March 31,	
	2018	2017
GAAP selling, general and administrative expenses	43,341	26,216
Stock-based compensation expense	(8,466)	(3,838)
Depreciation and amortization expense	(1,188)	(1,125)
Restructuring expense	—	(166)
Non-GAAP selling, general and administrative expenses (1)	33,687	21,087

(1) Commencing in the first quarter of 2018, the Company has excluded interest expense (income), net, and depreciation and amortization expense from the computation of its non-GAAP financial measures. The Company has revised prior year presentation in the tables above in order to conform to the current year presentation.

Sarepta Therapeutics, Inc.
Consolidated Balance Sheets
(unaudited, in thousands, except share and per share data)

	As of March 31, 2018	As of December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 557,234	$ 599,691
Short-term investments	491,757	479,369
Accounts receivable	39,848	29,468
Inventory	99,375	83,605
Other current assets	31,203	36,511
Total current assets	1,219,417	1,228,644
Property and equipment, net of accumulated depreciation of $19,817 and $18,022 as of March 31, 2018 and December 31, 2017, respectively	53,927	43,156
Intangible assets, net of accumulated amortization of $4,659 and $4,145 as of March 31, 2018 and December 31, 2017, respectively	14,473	14,355
Other assets	12,466	21,809
Total assets	$ 1,300,283	$ 1,307,964
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 17,379	$ 8,467
Accrued expenses	65,648	68,982
Current portion of long-term debt	3,446	6,175
Other current liabilities	4,723	4,708
Total current liabilities	91,196	88,332
Long-term debt	427,365	424,876
Deferred rent and other	4,962	5,539
Total liabilities	523,523	518,747
Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 99,000,000 shares authorized; 65,493,293 and 64,791,670 issued and outstanding at March 31, 2018 and December 31, 2017, respectively	7	6
Additional paid-in capital	2,029,767	2,006,598
Accumulated other comprehensive loss	(643)	(379)
Accumulated deficit	(1,252,371)	(1,217,008)
Total stockholders' equity	776,760	789,217
Total liabilities and stockholders' equity	$ 1,300,283	$ 1,307,964

Source: Sarepta Therapeutics, Inc.

Media and Investors:
Sarepta Therapeutics, Inc.
Ian Estepan, 617-274-4052
iestepan@sarepta.com

or

W2O Group
Brian Reid, 212-257-6725
breid@w2ogroup.com

Eteplirsen

AVI BioPharma
CHMP Oral Explanation
Date: 24 April 2018

Attendees

Name	Position
AVI BioPharma	
Douglas Ingram	President & Chief Executive Officer
Shamim Ruff, MSc	Sr. Vice President, Chief Regulatory Affairs Officer
Helen Eliopoulos, MD	Executive Director, Regulatory Affairs
Diane Frank, PhD	Sr. Director, Translational Development
Chris Mix, MD	VP, Clinical Development
Heidi Krenz, MD	VP, Pharmacovigilance
Alex Meldrum	*Senior Consultant Regulatory Affairs*
External Experts	
Craig M. McDonald, MD	*Study Chair, CINRG Duchenne Natural History Study; Professor and Chair, Dept. Physical Medicine & Rehabilitation; Director, Neuromuscular Disease Clinics; University of California at Davis School of Medicine*
Bernard Kinane, MD	*Chief, Pediatric Pulmonary Unit, Massachusetts General Hospital for Children*
Ping-Yu Liu, PhD	*Statistician, Fred Hutchinson Cancer Research Center, Member Emeritus*

Proposed Indication for DMD

- **DMD is a rare, fatal disease**
  - Result from inability to produce dystrophin
- **Proposed indication is in a subpopulation of DMD**
  - Treatment of Duchenne muscular dystrophy (DMD) in ambulatory patients 4 years and older who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping
- **No approved therapy for this population in Europe**

What You Will Hear Today

- **Eteplirsen:**
  - Induces exon skipping and produces functional dystrophin
  - Consistently slows disease progression
    - Multiple studies and endpoints
    - 115 DMD subjects ([redacted] of the eligible population in Europe)
  - Favorable safety profile and well tolerated
- **Commitment to a confirmatory trial if granted conditional marketing approval**
- **Urgent unmet need**

Dystrophin Quantification:

Proof of Eteplirsen Mechanism of Action

Craig M. McDonald, MD

Study Chair, CINRG Duchenne Natural History Study
Professor and Chair, Dept. Physical Medicine & Rehabilitation
Professor of Pediatrics
Director, Neuromuscular Disease Clinics
University of California at Davis School of Medicine

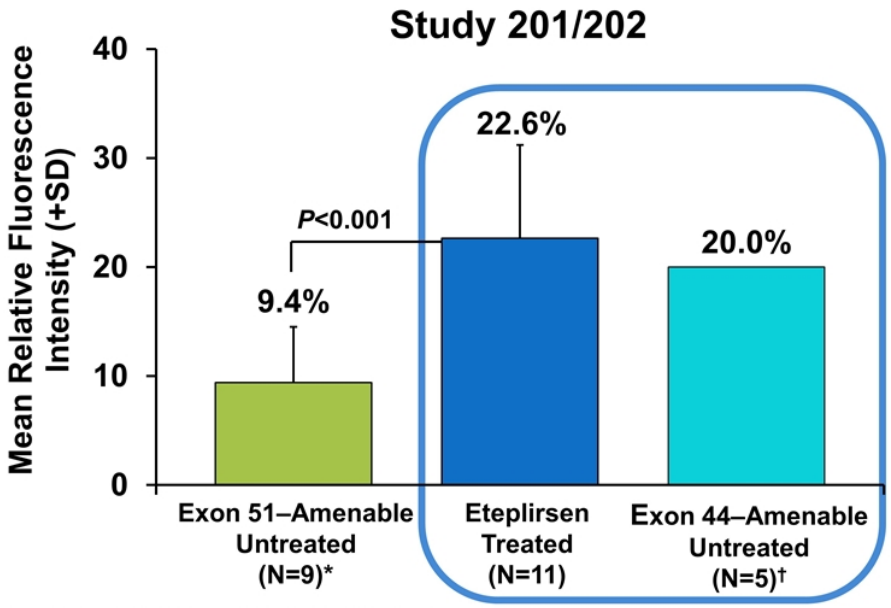
Goal of Eteplirsen: Production of Functional Truncated Dystrophin Protein

- **The truncated dystrophin proteins produced by eteplirsen are functional and the *same proteins* produced from conception in Becker muscular dystrophy patients (near normal life expectancy)**
- **The goal of eteplirsen is not to produce a Becker phenotype**
- **The goal is to slow disease progression by producing low levels of dystrophin (as in exon 44 skip amenable DMD patients)**

What Amount of Dystrophin is Clinically Relevant?

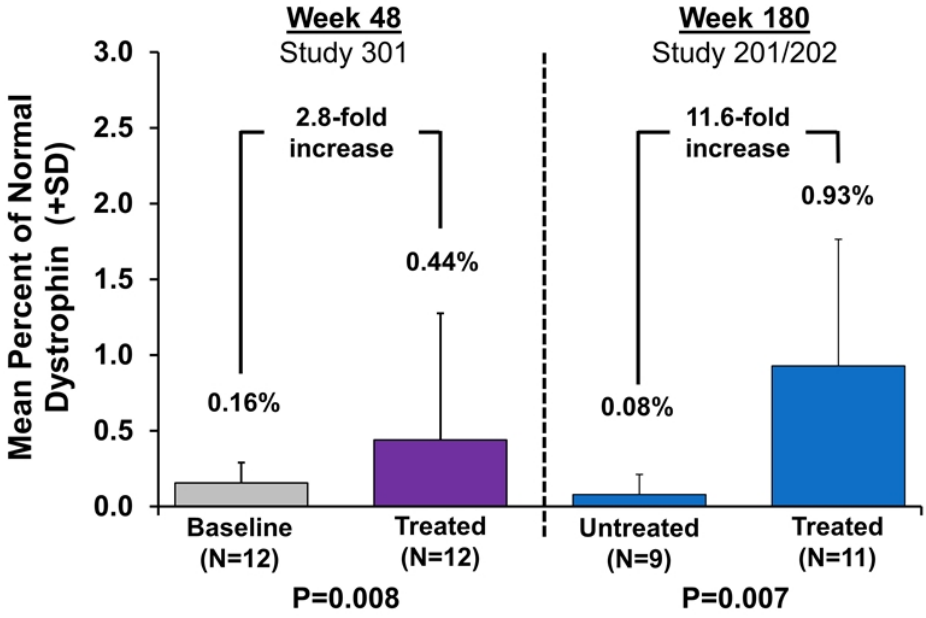
- **Low levels of truncated dystrophin are naturally present in exon 44 skip amenable patients with DMD – *an experiment in nature***
- **Exon 44 skip amenable patients have levels of dystrophin which are similar to those seen with pharmacologic exon skipping (20% by IHC)**
- **These levels of dystrophin are associated with a milder phenotype (based on 6MWT, NSAA, and loss of ambulation)**
  - 50% of exon 44 skip amenable patients ambulate beyond 20 years

Similar Quantity of Truncated Dystrophin Protein Seen in Both Eteplirsen Treated Patients and Exon 44 Amenable Patients



*Untreated Exon 51-amenable EC for Study 201/202 Week 180.
†Untreated Exon 44-amenable from Anthony K, et al. Neurology. 2014;83(22):2062-2069.

Eteplirsen Produces Dystrophin Which Accumulates Over Time Validated Western Blot



Major Objections Related to Dystrophin

Objection	Eteplirsen MAA
◆ The very modest increase in dystrophin production (mean 0.93% WB of normal dystrophin in study 201/202 and 0.44% in study 301)	✓ Clinical relevance of the dystrophin produced is confirmed ✓ Levels observed in Study 201/202 are similar to exon 44 skip amenable
◆ No increase was detected in a number of patients in both studies, only partially supports the proposed mechanism of action of eteplirsen	✓ Western Blot shows a range of response, as expected ✓ All evaluated patients (N=36) have demonstrated exon skipping by RT-PCR

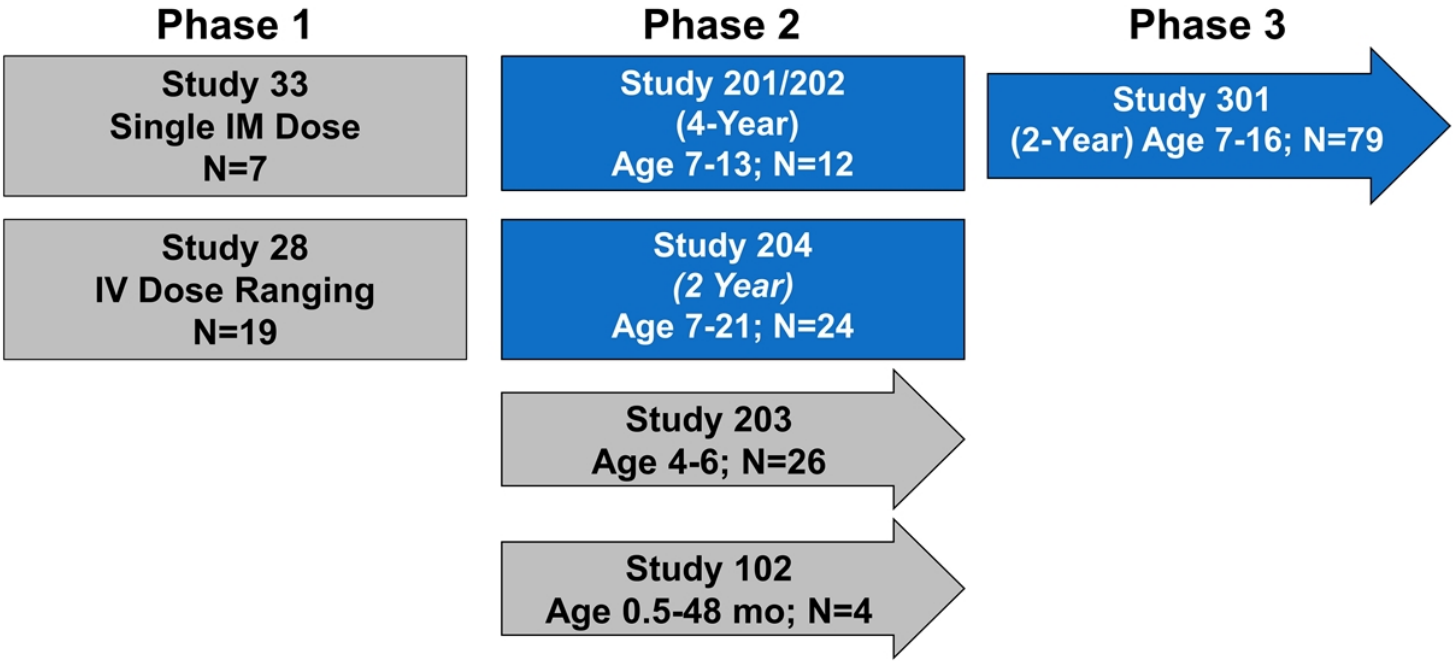
8 Year Old Untreated DMD Patient: Typical Attempted Run Steroid Treated Since Age 4; 6MWD = 330 Meters

Review of Clinical Data

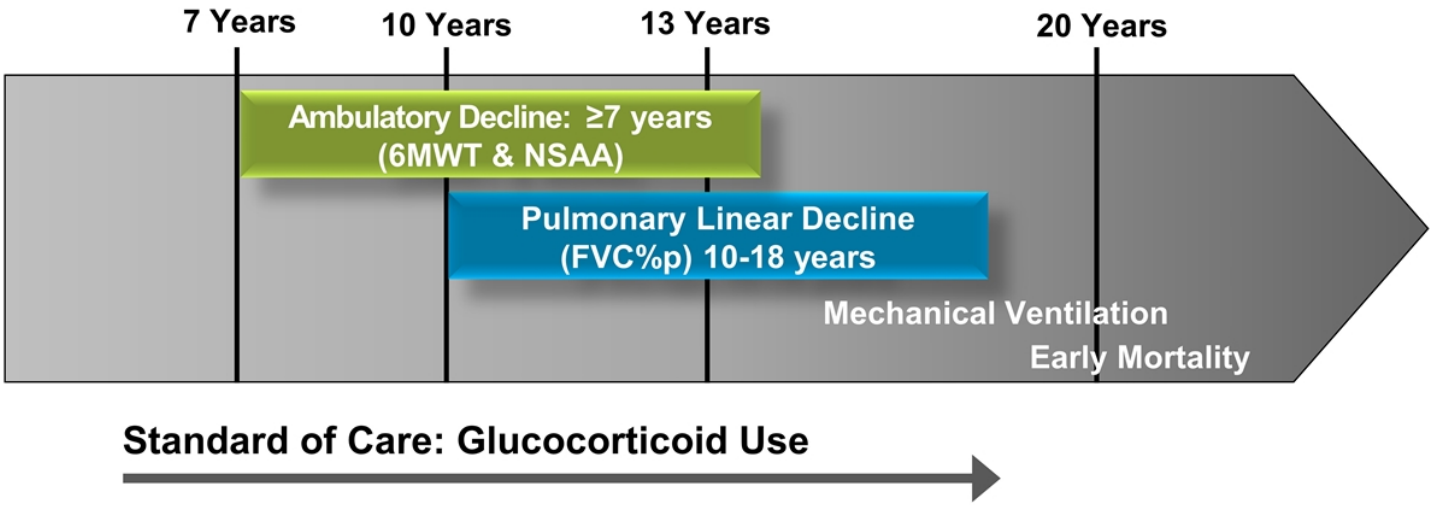
Helen Eliopoulos MD

Executive Director, Regulatory Affairs

Eteplirsen DMD Clinical Program: 3 Trials Contribute to Efficacy



Clinical Efficacy Focuses on Phases of Decline in DMD



Standard of Care: Glucocorticoid Use

Rigorous Identification of Most Appropriate External Controls

- **12 DMD Registries and Untreated Controls identified**
- **Only 3 of 12 prospectively collected ambulatory or pulmonary endpoints**
  - Long term data for endpoints
  - Patient level data available for genotyping

Registry (Patients)	Six Minute Walk Test (6MWT)	North Star Ambulatory Assessment (NSAA)	Loss of Ambulation (LOA)	Forced Vital Capacity %predicted (FVC %p)
Italian Telethon (N=96)	✓	✓	✓	
Leuven NMRC (N=89)	✓		✓	
CINRG* (N=397)			✓	✓

*At the time of data collection CINRG had limited longitudinal 6MWT & NSAA data.

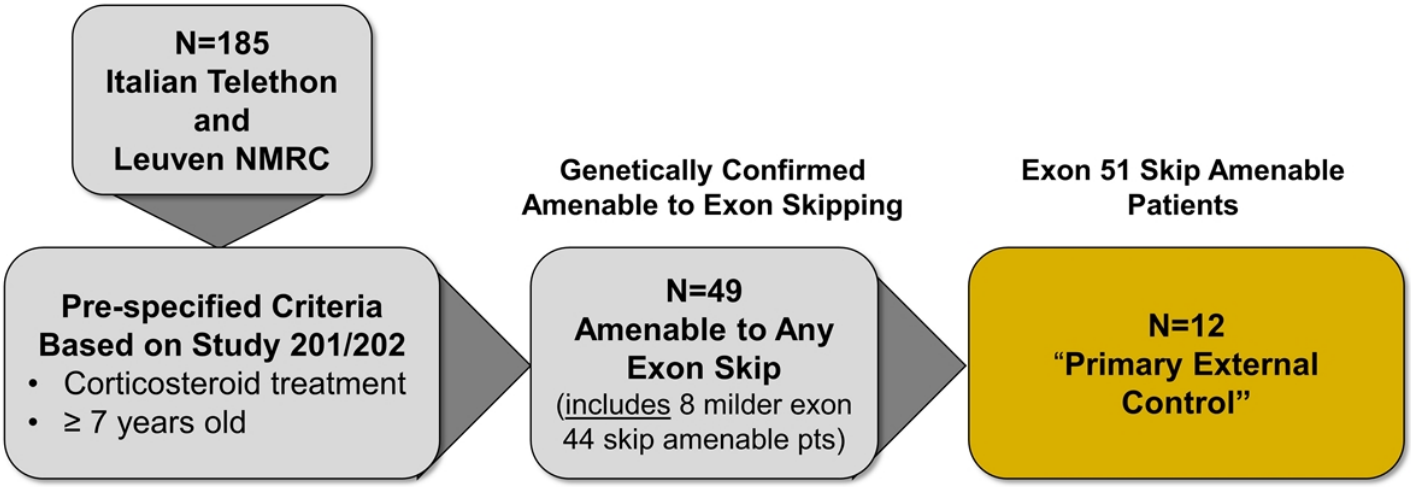
Ambulatory Data vs Primary EC

Six-Minute Walk Test (6MWT)

Loss of Ambulation (LOA)

North Star Ambulatory Assessment (NSAA)

Identification of Primary External Control (EC) for Ambulatory Outcomes from Italian Telethon and Leuven Centers



Primary EC Comparable to Studies 201/202 & 301

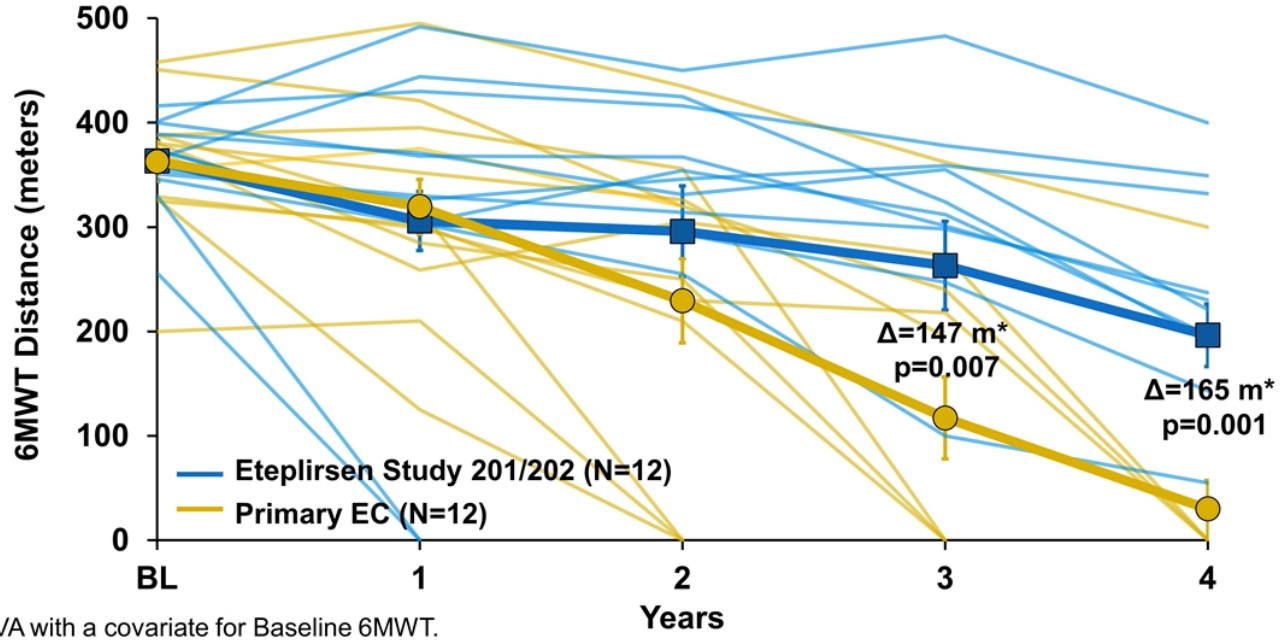
Key Baseline Characteristics

Parameter	Primary EC N=12	Study 201/202 N=12	Study 301 N=60
Mean Age (years)	9.5	9.4	9.6
Mean 6MWT (m)	362.8	363.2	376.2
Mean NSAA	22.8	24.9	21.9
Corticosteroid Type			
Deflazacort	8 (67%)	8 (67%)	17 (28%)
Prednisone	4 (33%)	4 (33%)	43 (72%)

Primary EC and Study 201/202 Received High Standard of Care

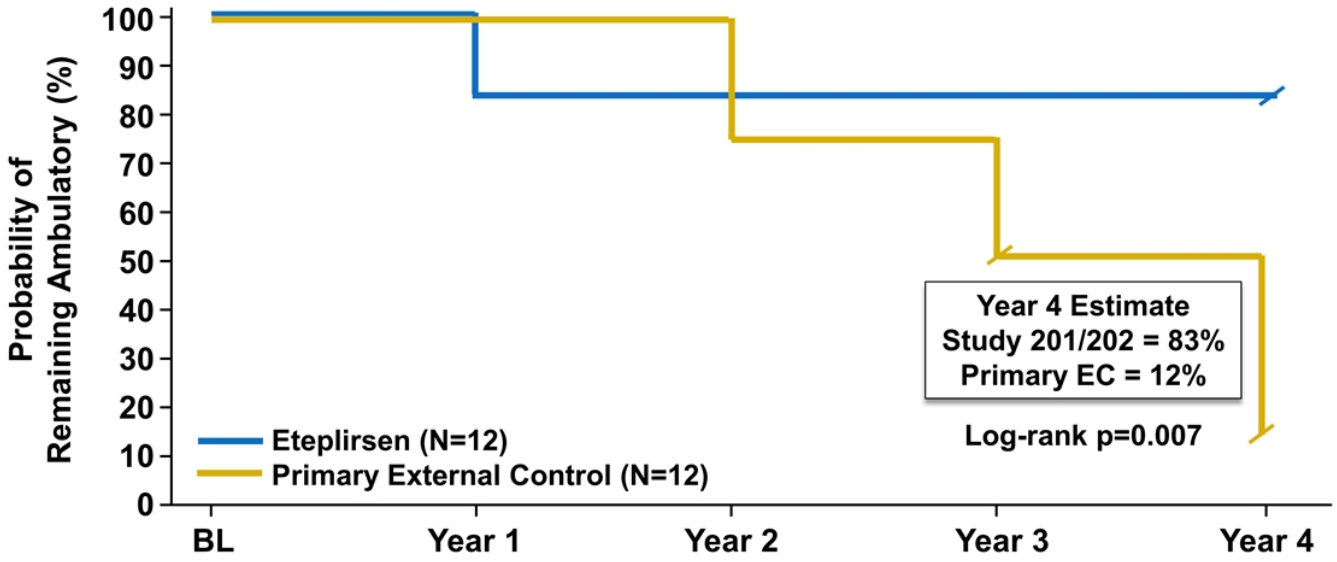
Frequency of Physical Therapy (Days/wk or Usage)	Primary EC: N=12 (Patients)	Study 201/202 N=12 (Patients)
4-6	4	2
2-3	8	3
≤1	0	5
None	0	2

Study 201/202 vs Primary EC: Individual 6MWT Over 4 Years



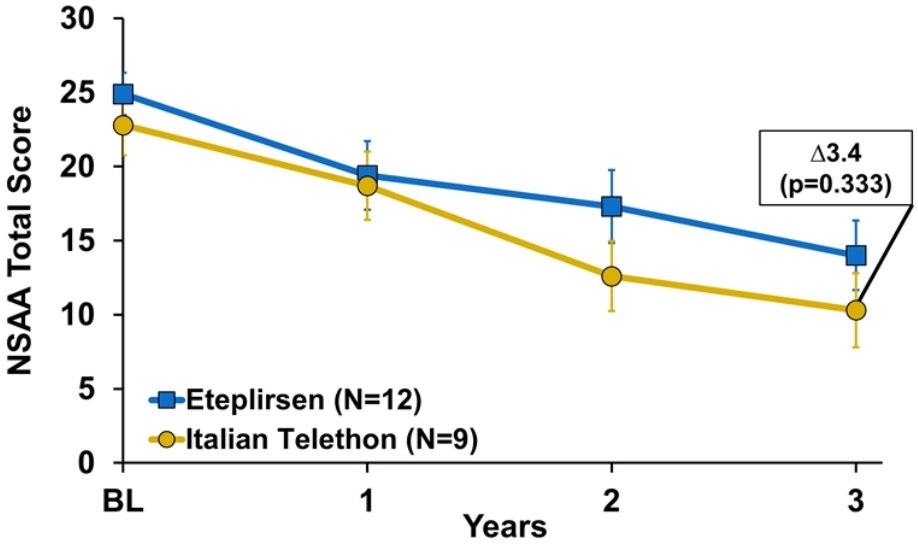
ANCOVA with a covariate for Baseline 6MWT.
*Δ= Difference in Mean Change from Baseline.

Probability of Remaining Ambulatory
Kaplan-Meier Estimates for Study 201/202 vs Primary EC



Study 201/202	12	10	10	10	10
Primary EC	12	12	9	6	1

Eteplirsen Study 201/202 vs Italian Telethon: Slowing of NSAA Directionally Consistent with 6MWT



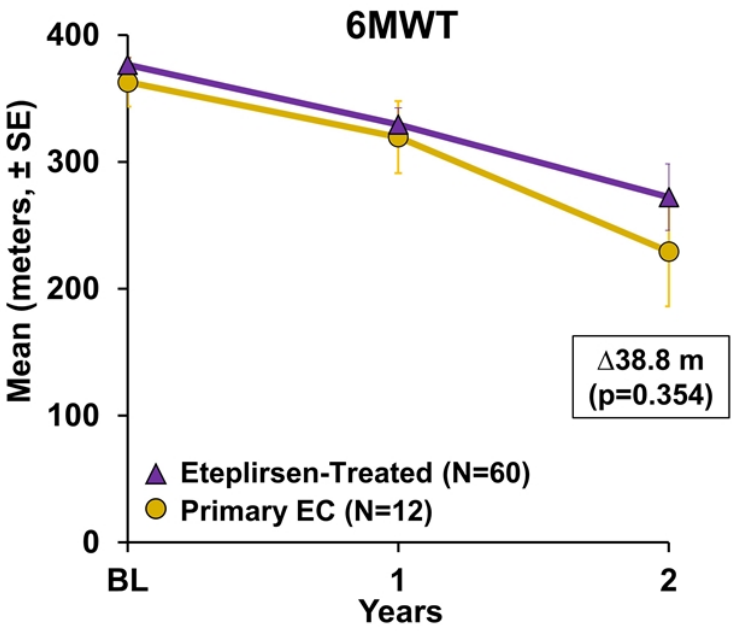
#	NSAA Activity
1	Stand
2	Walk
3	Stand from chair
4/5	Stand on one leg (right/left)
6/7	Climb box step (right/left)
8/9	Descend box step (right/left)
10	Sit from supine
11	Rise from supine (Gower)
12	Lift head from floor
13	Stand on heels
14	Jump
15/16	Hop on one leg (right/left)
17	10 meter run/walk

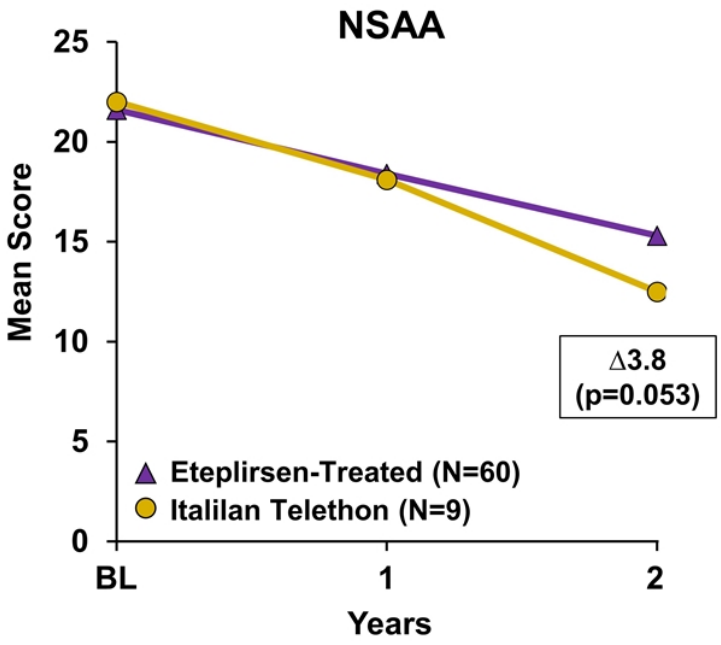
Δ = Difference in mean change from baseline.

ICH E10 (2.5.4 Validity of Inference from External Controls)

Externally Controlled Trials Can be Persuasive:	Comparison of Study 201/202 vs Primary EC
◆ Disease has a well documented, highly predictable course	✓ DMD declines are well characterized for homogenous populations
◆ Covariates influencing the outcomes of disease are well characterized	✓ Key baseline characteristics comparable (age, 6MWT, steroids)
◆ Observational variables are similar	✓ SOC comparable (physical therapy, steroids)
◆ Study endpoint is objective	✓ Loss of ambulation is an objective endpoint
◆ Treatment outcome is markedly different	✓ Large magnitude of effect over 4 years

Study 301 Interim Analysis of 6MWT and NSAA vs Primary EC: Directionally Favoring Eteplirsen Patients at Year 2





Pulmonary Data vs CINRG Control:

Forced Vital Capacity % Predicted (FVC%p)

(% FVC in healthy individuals normalized to age & height)

CINRG Duchenne Natural History Study

Registry (# of Patients)	FVC%p
Italian Telethon (N=96)	
Leuven NMRC (N=89)	
CINRG (N=397)	✓

- **Global (20 Centres) – 3 in EU (Sweden, Italy and UK)**
- **Study Period: 2006 – Present**
- **Largest prospective multicenter DMD natural history cohort**
  - N= 397 patients with pulmonary data

CINRG website. www.cinrgresearch.org/. Accessed March 17, 2018.

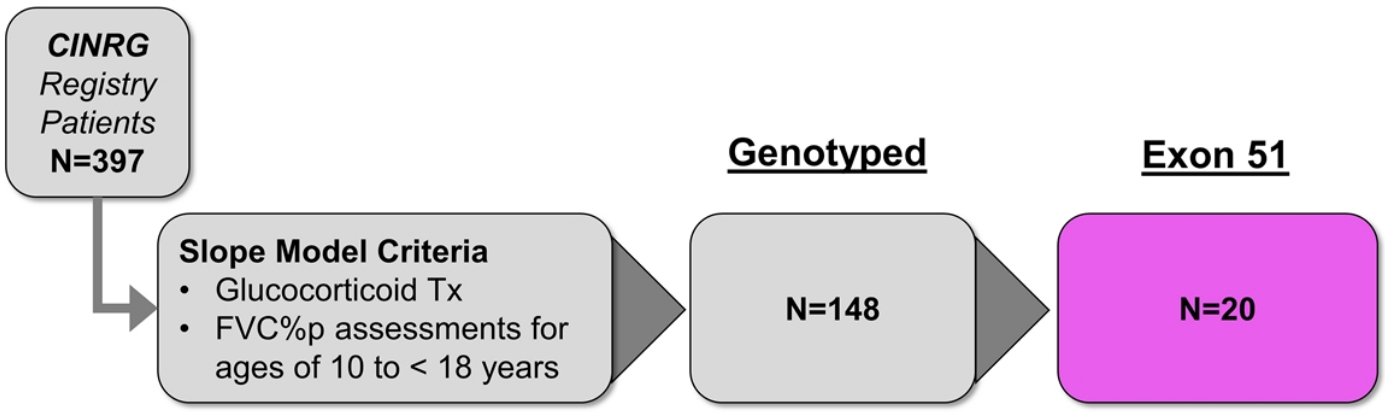
Eteplirsen Pulmonary Outcomes Evaluated in 2 Ways Addressing CHMP Feedback

1. **Slope Model: FVC%p annual change *by age (10 to <18 years)***
   - Studies 201/202, 301 & 204
   - Evaluated eteplirsen effects on patients who are expected to decline

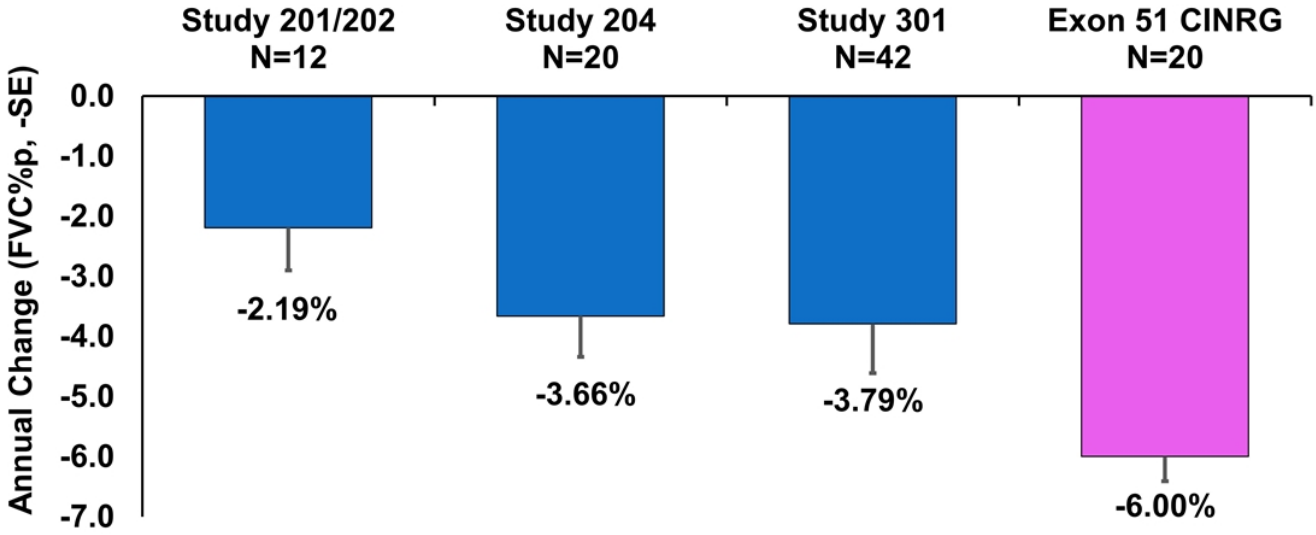
2. **Time on Study 201/202: Change of FVC%p *over 4 years***

Statistical analyses were pre-specified prior to accessing CINRG patient level data

Identification of CINRG Comparator for Slope Model Analysis *(Ages 10 to < 18 years)*

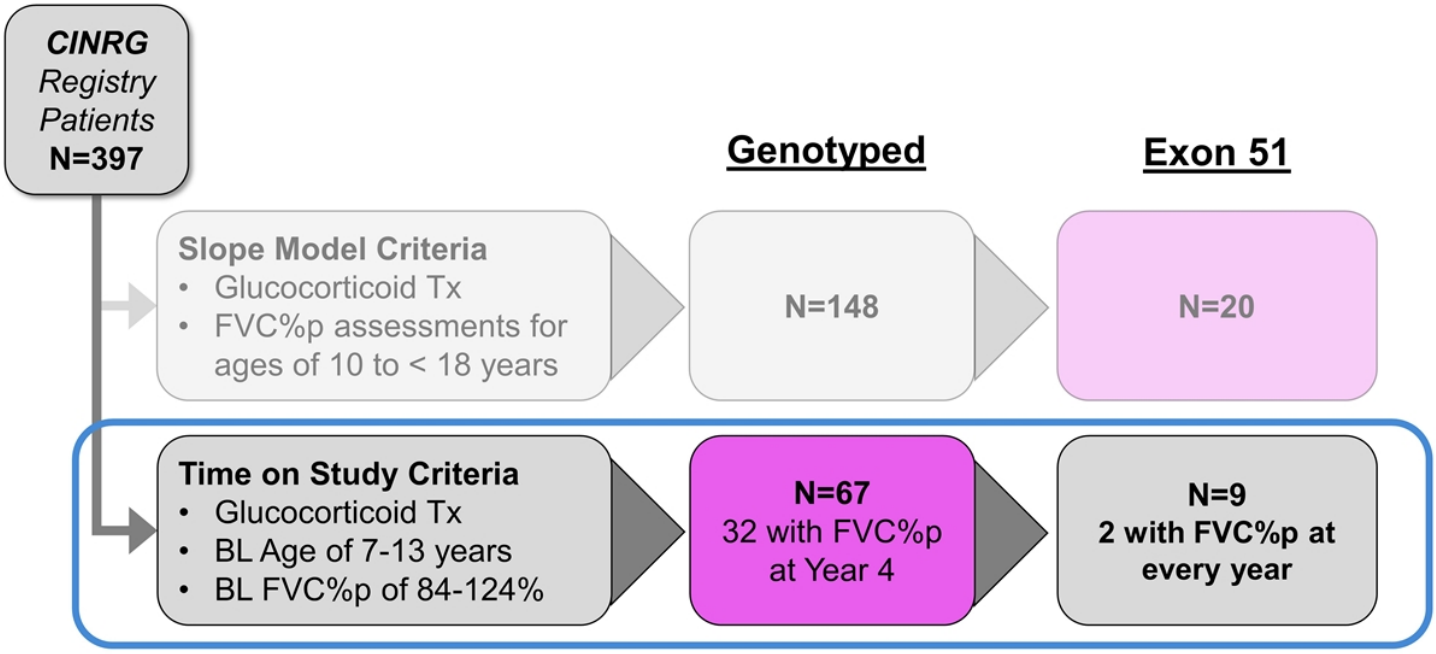


Slope Model: Ages 10 to <18 yrs vs Exon 51 CINRG EC

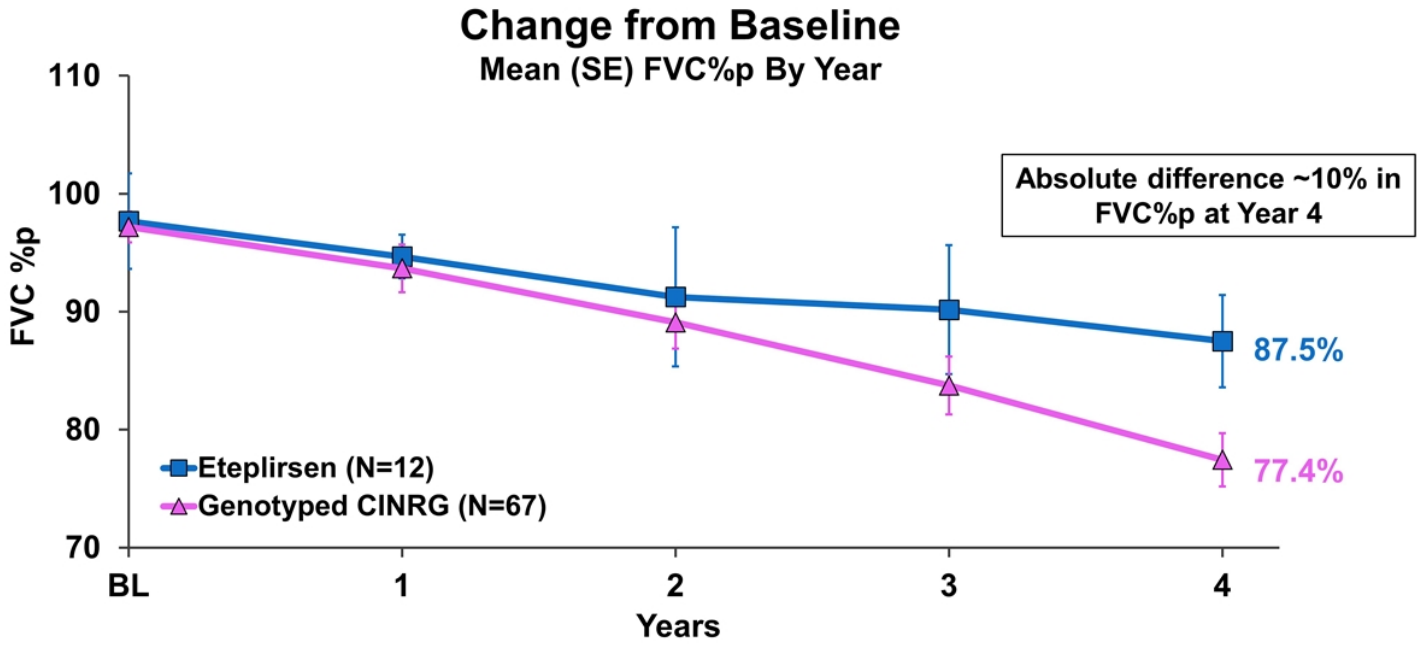


P-value vs CINRG	< 0.001	0.004	0.017	N/A

Identification of CINRG Comparator for Time on Study 201/202 Over 4 Years



Time on Study 201/202: Change of FVC%p vs Genotyped CINRG EC (N=67) Patients Aged 7-13 Years



Clinical Efficacy Has Been Convincingly Demonstrated

Major Objections	Eteplirsen MAA
◆ Evidence from the 24 week placebo control period of Study 201 is weak	◆ Short treatment duration of placebo did not enable differentiation of effect
◆ Study 201/202 and interim analysis of 301 compared with external control groups is very weak	✓ Large magnitude of effect for Study 201/202 ✓ Ambulatory data from Study 301 (N=60) are supportive ✓ Significant slowing of pulmonary decline in 3 studies (N=74)
◆ Long-term, compelling data are essential to elucidate whether eteplirsen has a benefit	✓ Study 201/202 shows a consistent temporal pattern over 4 years ✓ Gradual accumulation of dystrophin

Eteplirsen Safety Experience is Favorable and Generally Consistent with Pediatric DMD

- **171 clinical patients* (309 patient-years at ≥30 mg/kg); 106 patients with ≥ 2 years**
  - Infusion-related reactions; most commonly mild headache, rash, vomiting
  - 1 SUSAR urticaria, resolved with no recurrence
- **[redacted] post-marketing patients ([redacted] patient-years):**
  - Safety profile generally similar to clinical trials
  - Infusion-related reactions, including 2 bronchospasm events which resolved
- **No signal for risks observed with drisapersen**
  - No hepatotoxicity, renal toxicity, hematologic toxicities, severe cutaneous reactions

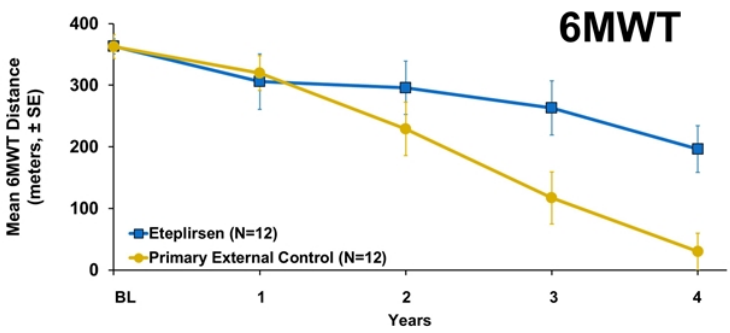
**Includes all doses and routes of administration.*

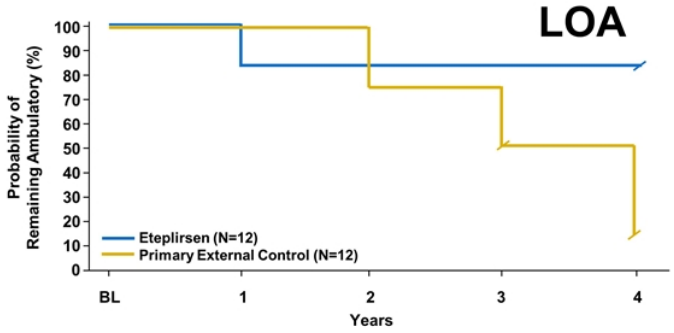
Benefit Risk of Eteplirsen

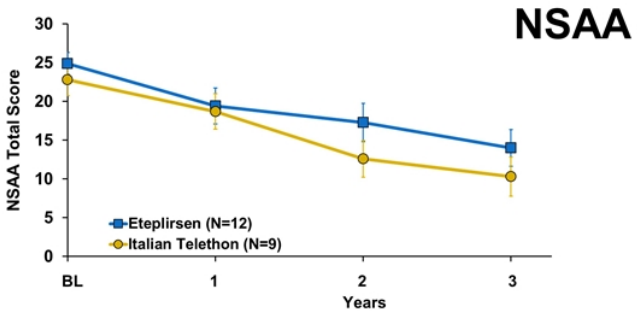
Craig M. McDonald, MD

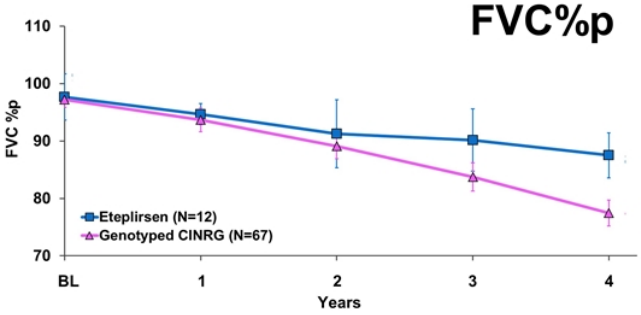
Study Chair, CINRG Duchenne Natural History Study
Professor and Chair, Dept. Physical Medicine & Rehabilitation
Professor of Pediatrics
Director, Neuromuscular Disease Clinics
University of California at Davis School of Medicine

Study 201/202: Eteplirsen Slows Disease Progression Across Multiple Endpoints

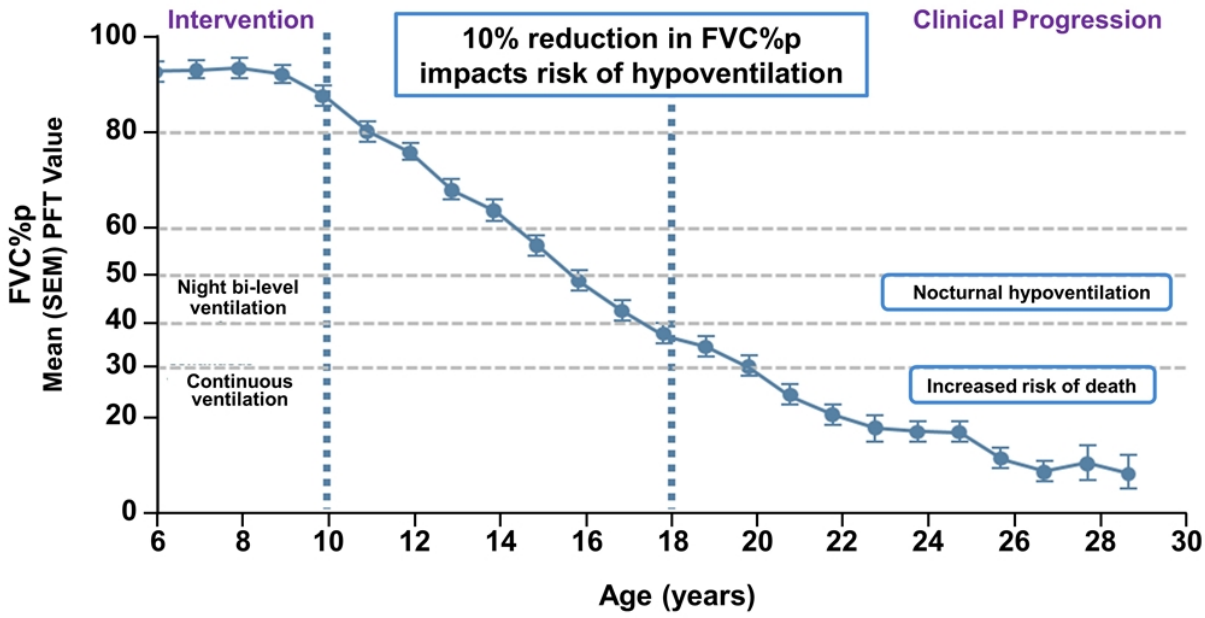






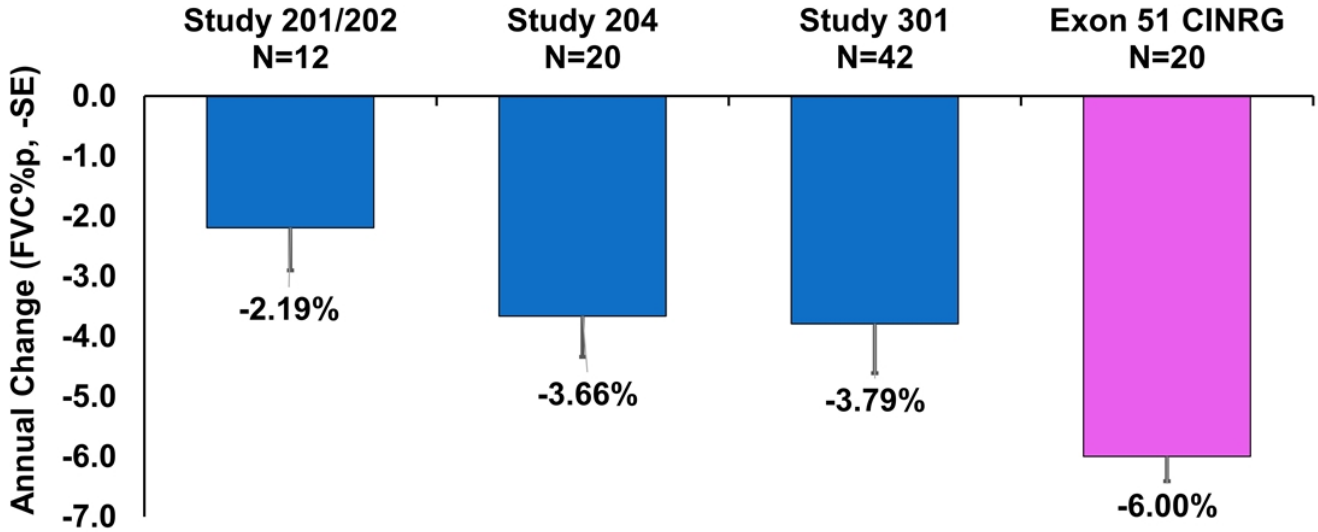


Declining FVC%p Linked to Clinically Meaningful Thresholds and Risk of Death (Based on CINRG Data)



Modified from †Mayer, et al. 2017; CINRG Data: McDonald CM, et al. 2018.

Eteplirsen Slows the Rate of Pulmonary Decline Across Three Studies



P-value vs CINRG	< 0.001	0.004	0.017	N/A

Clinical Experience with Eteplirsen (Prof. McDonald): Three Youngest Patients Treated for > 3 Years

7.5 yrs (Study 203) | 8.5 yrs (Study 203) | 10 yrs (Study 301)

Questions

Shamim Ruff, MSc (Moderator)

Sr. Vice President, Chief Regulatory Affairs Officer

Backup Slides Displayed at Oral Explanation Q&A

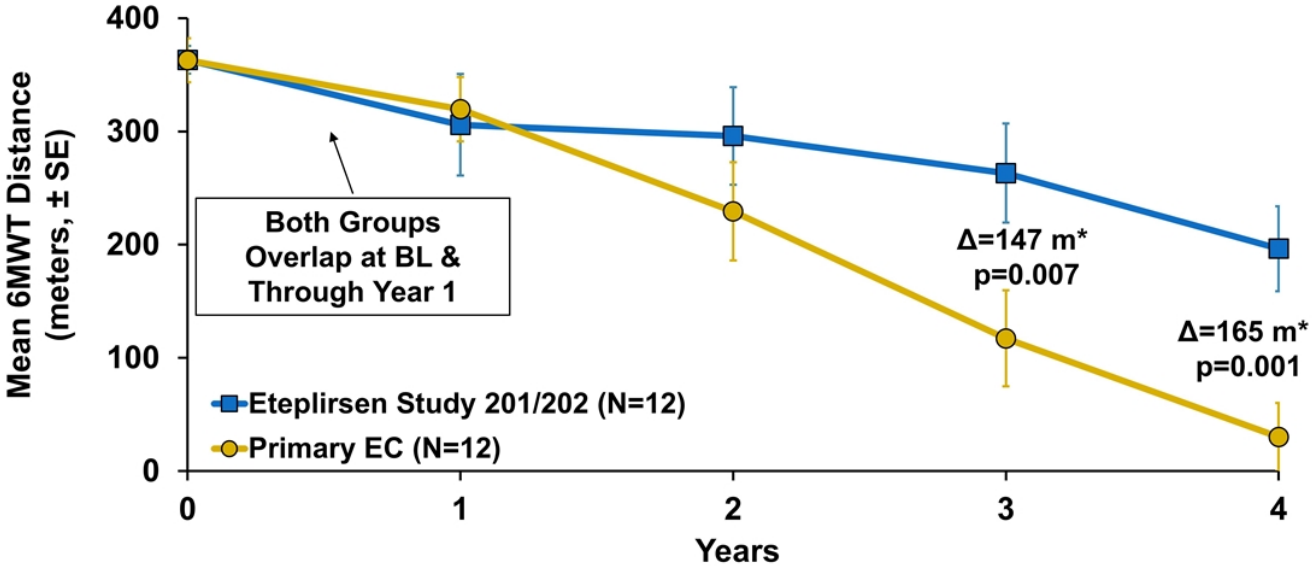
Study 201/202: Similar Eteplirsen Benefit over Primary Comparator, independent of age of steroid initiation and regimen

Parameter	Eteplirsen Combined 201/202 Studies (N=12)	Primary External Control (amenable to Exon 51 skipping) (N=12)
Age at Steroid Start	5.2	6.5
Corticosteroid Type		
Deflazacort	8 (67%)	8 (67%)
Prednisone	4 (33%)	4 (33%)
Corticosteroid Regimen		
Continuous	11 (92%)	8 (67%)
Intermittent	1 (8%)	4 (33%)

6MWT (m) difference from Primary EC at Year 4	
Primary ANCOVA adjusted for BL 6MWT	165, p<0.001
ANCOVA adjusted for BL 6MWT, Age, **Age at Steroid Start**	163, p=0.005
MMRM adjusted for BL Age, 6MWT & **Corticosteroid Type**	154, p<0.001
ANCOVA adjusted for BL 6MWT, **Corticosteroid Regimen**	157, p=0.003

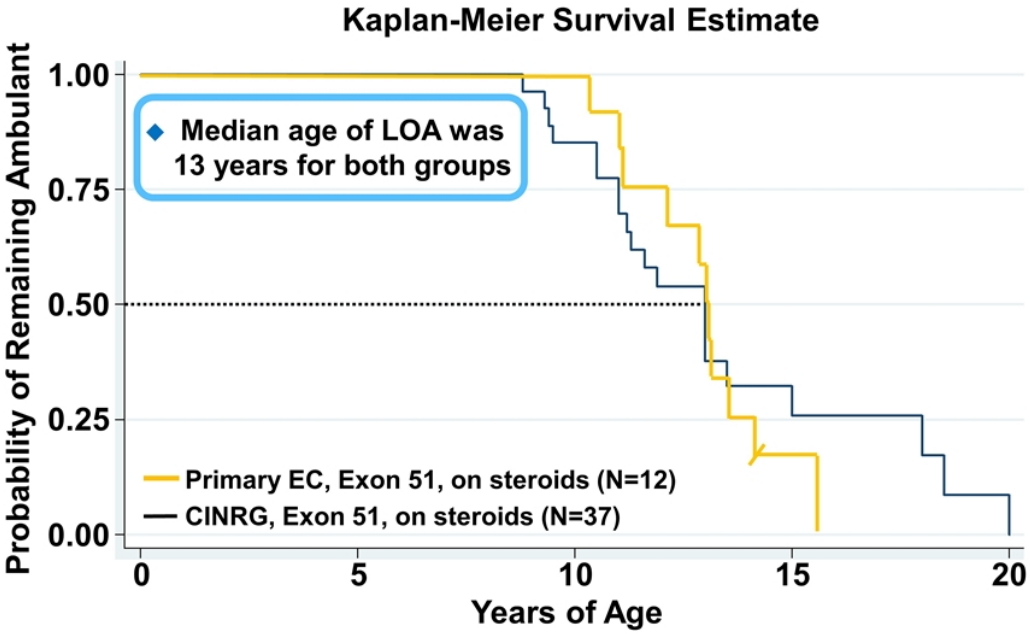
- Slightly younger age of steroid initiation and higher proportion of continuous regimen for eteplirsen Sensitivity analyses adjusting for these covariates showed >150 meters with nominal significance

6MWT Study 201/202 vs Primary EC: Significant Benefit at Years 3 and 4



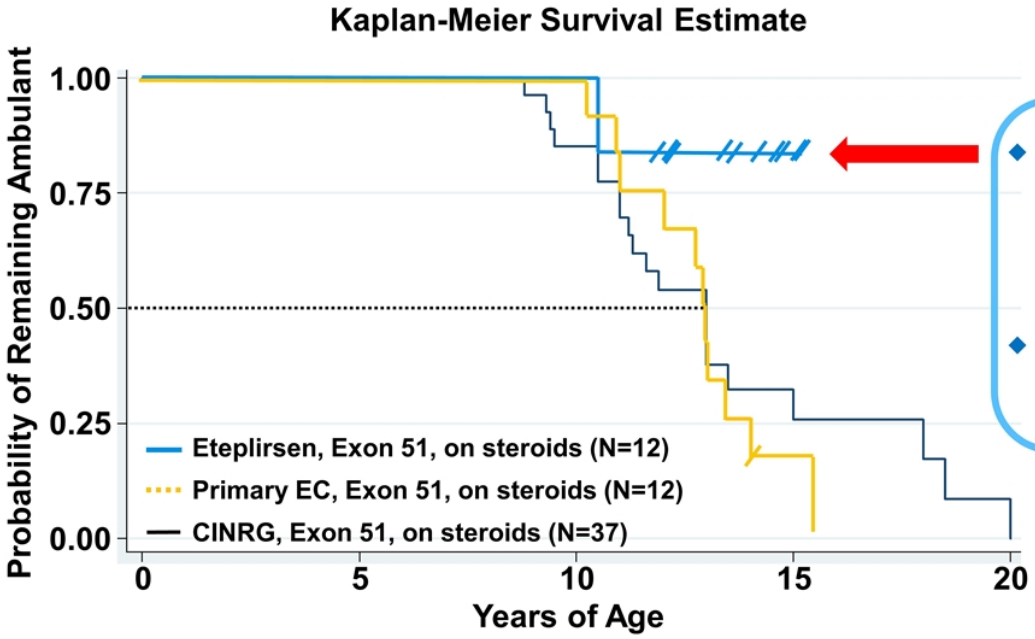
ANCOVA with a covariate for Baseline 6MWT.
*Δ= Difference in Mean Change from Baseline.

Primary EC Comparable to CINRG Exon 51 Cohort on LOA



Data provided by CINRG and Primary External Control.

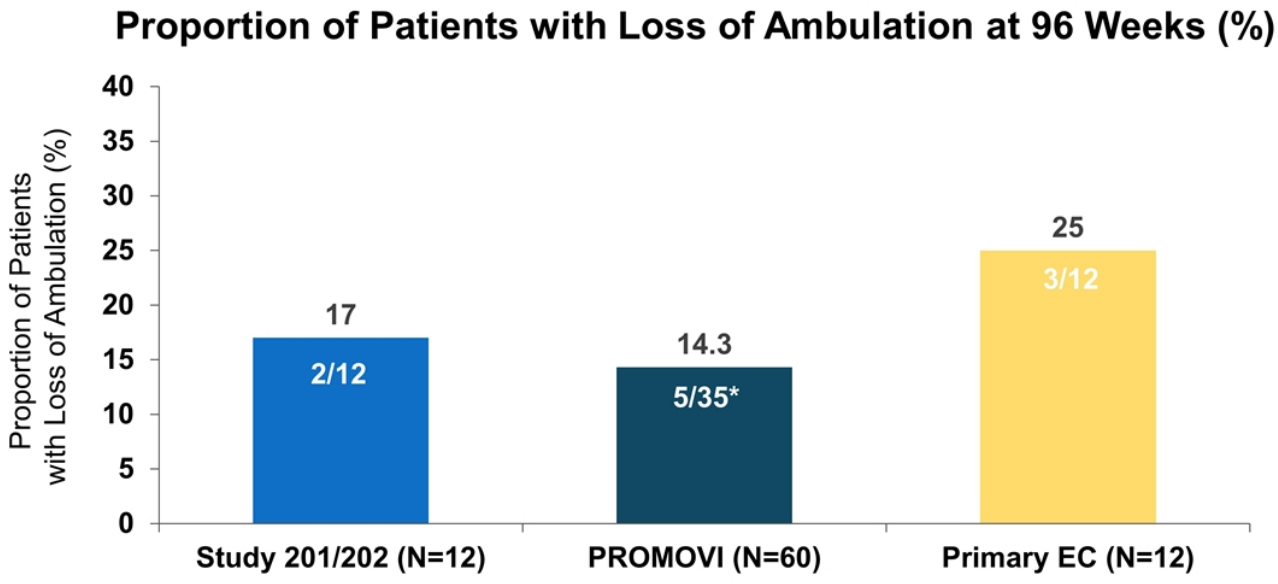
Eteplirsen Preserves Ambulation: Comparison to EC and CINRG Exon 51



- Eteplirsen Median age of LOA not reached
  - Median Age of N=10 ambulant patients at last 6MWT: 14.0
- CINRG Median age of LOA was 13.0
  - 95% CI: 11.5-15.0

Data provided by CINRG and Primary External Control.

Proportion of Patients Who Lost Ambulation Was Consistent Between Study 201/202 and 301



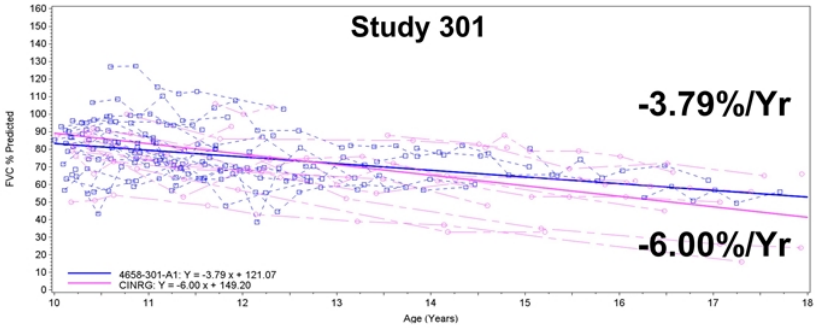
*Interim analysis. Only patients who completed full 96 weeks of eteplirsen treatment, with 6MWT were included in denominator (n=35).

Study 201/202 vs Primary EC
6MWT Adjusting for Ability to Rise, Rise Time

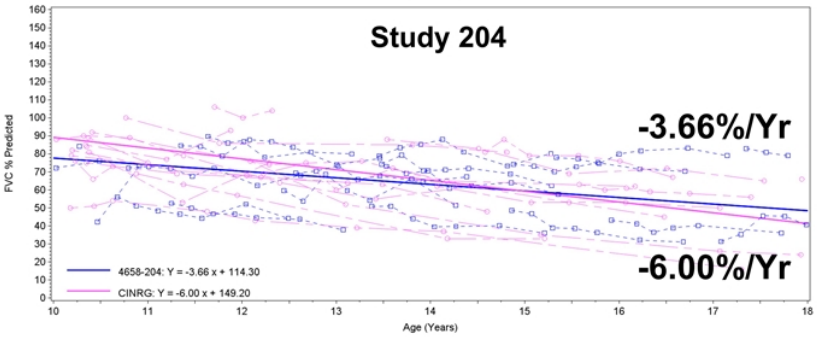
- For 201/202 vs Primary EC, there are only 1-2 patients in each group who were unable to rise independently at Baseline
- Therefore, an analysis adjusting for ability to rise was not feasible

- An analysis was conducted adjusting for baseline rise time which resulted in a difference of 180 meters (p=0.001)

Age-Based (10 to <18 years) Slope Model Analysis Confirms that Eteplirsen Attenuates the Rate of Pulmonary Decline



	Pts	# of Tests
Study 301	42	184
Exon 51 CINRG	20	88



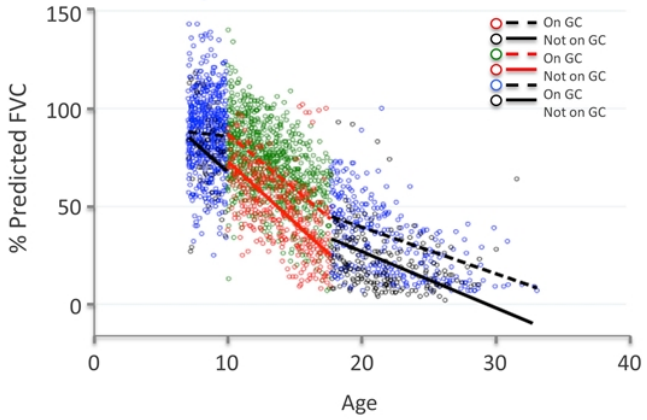
	Pts	# of Tests
Study 204	20	117
Exon 51 CINRG	20	88

Study 301: Eteplirsen Benefit over Biomarin Placebo – Pre-specified sensitivity analysis adjusting for steroid type

Parameter	Study 301 Primary Efficacy (N=59)	Biomarin Study 044 Placebo (N=20)
Age: Mean Min, Max	9.6 (7, 16)	8.8 (7, 12)
6MWT (m): Mean Min, Max	378 (301, 450)	368 (307, 437)
Steroid Type (n, %) DFZ PRD	 16 (29%) 43 (71%)	 11 (55%) 9 (45%)
6MWT difference from Biomarin Placebo (m) at Year 1	19.9 m (P=0.304)	

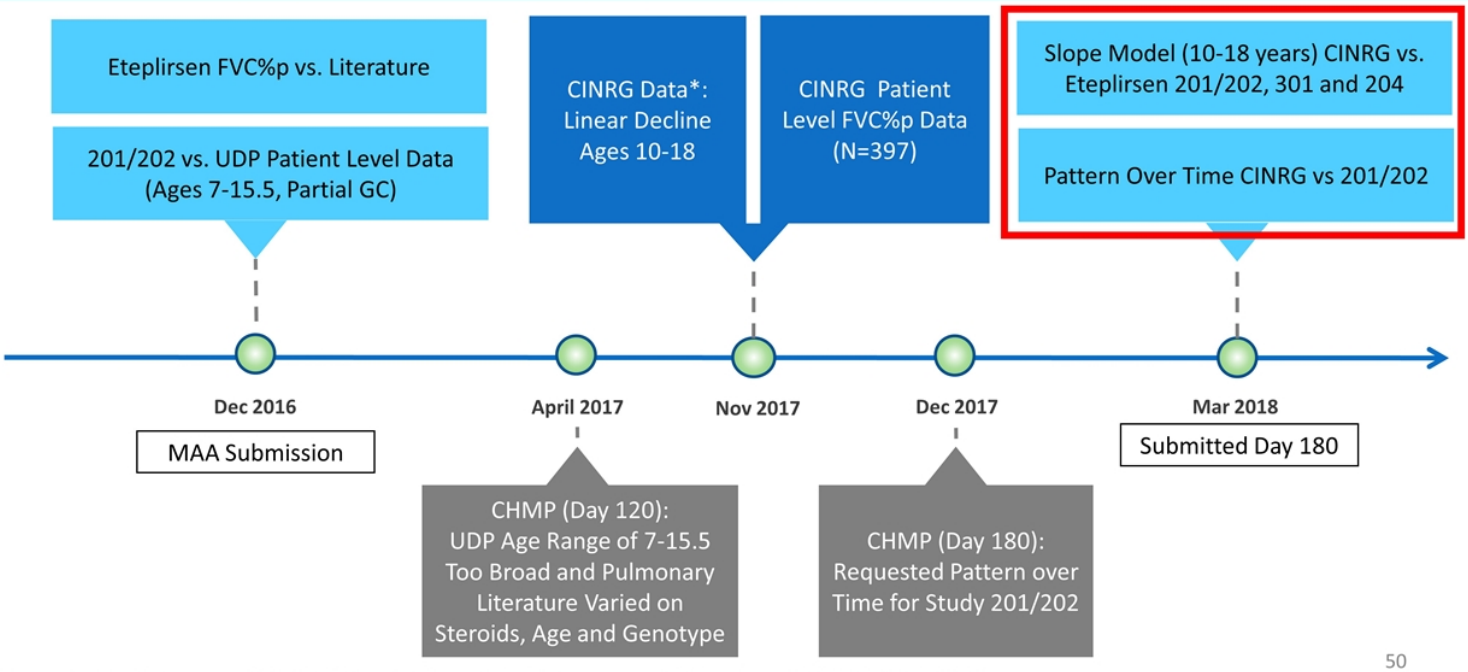
Contemporary Natural History Data for PFTs (CINRG, n=397)

Longitudinal Models in DMD Patients 7 Years and Older Over Time



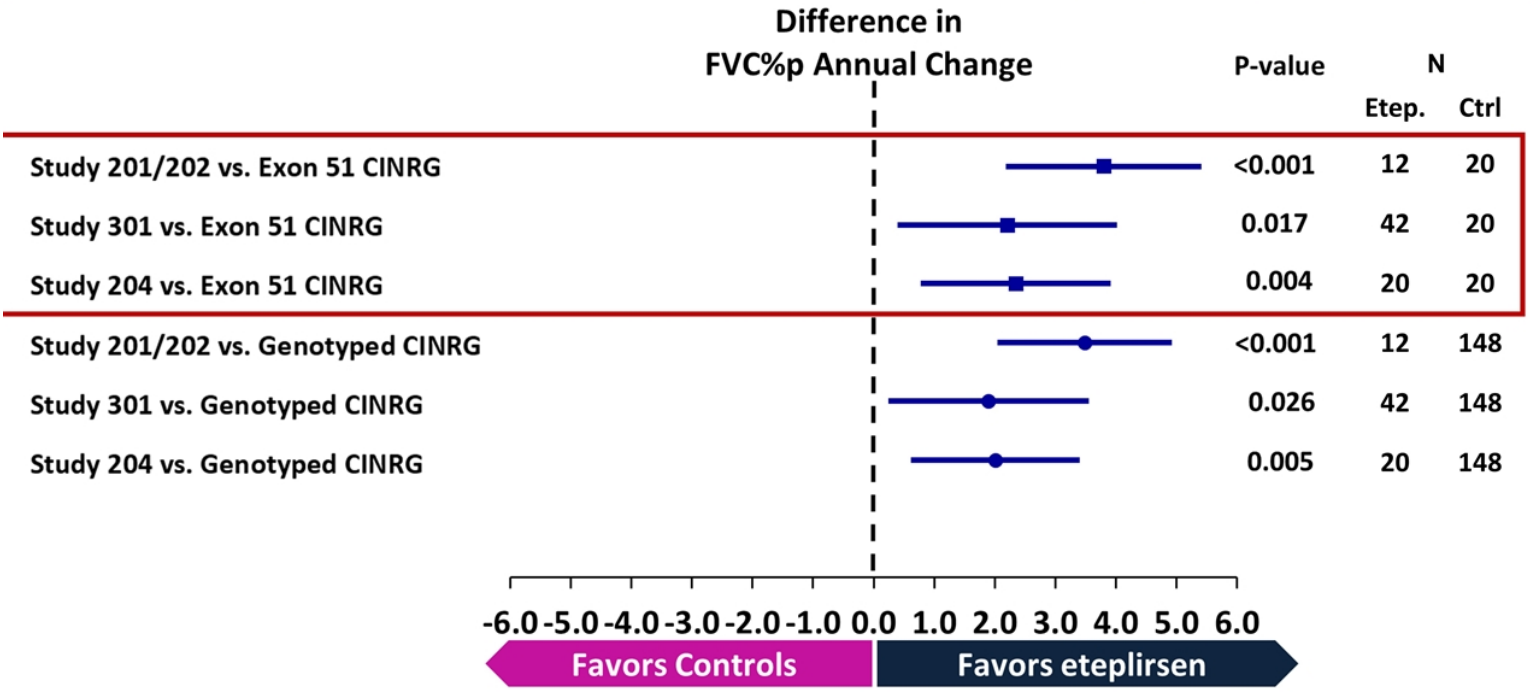
PFT assessment	GC use status	Change per age in 7 to 9.9 year olds		Change per age in 10 to 17.9 year olds		Change per age in those 18 and older	
		N (obs) N (indiv)	Coefficient (p-value)	N (obs) N (indiv)	Coefficient (p-value)	N (obs) N (indiv)	Coefficient (p-value)
FVC % predicted	Not on GC	63 35	-5.91 (0.002)	358 89	-6.06 (<0.001)	207 55	-2.78 (<0.001)
	On GC	567 168	-0.69 (0.23)	886 198	-5.44 (<0.001)	309 72	-2.30 (<0.001)

CINRG Comparators: Address CHMP Concerns Regarding Variable Literature

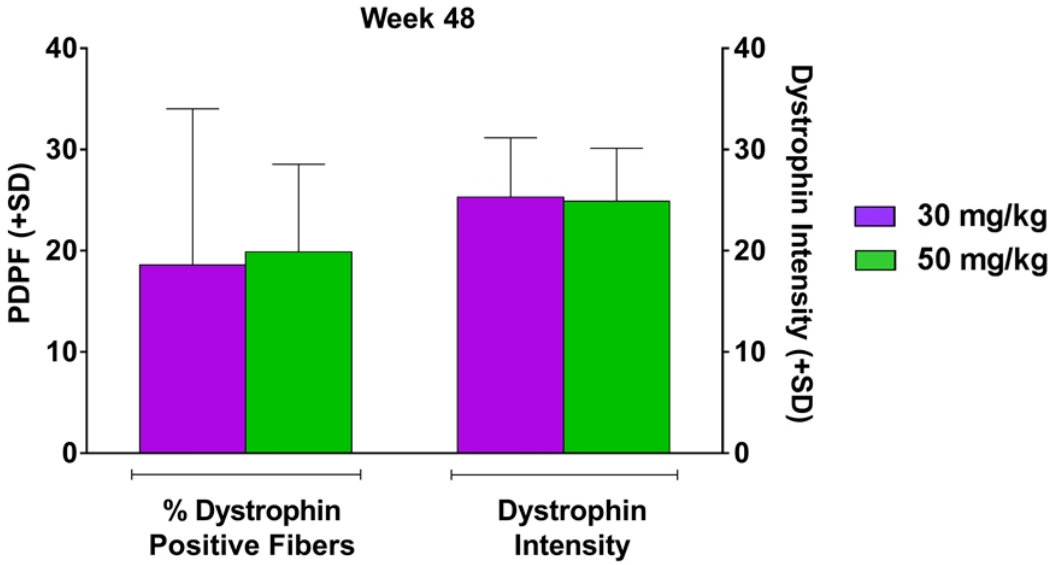


*Mayer et al., *US Neurology*, 2017;13(1):35–41, McDonald et al. *Neuromuscular Disorders*, 2017; 27(2): S115–S116

Difference in FVC%p annual change favors eteplirsen vs. external controls (Slope Models, Ages 10 - <18 years)

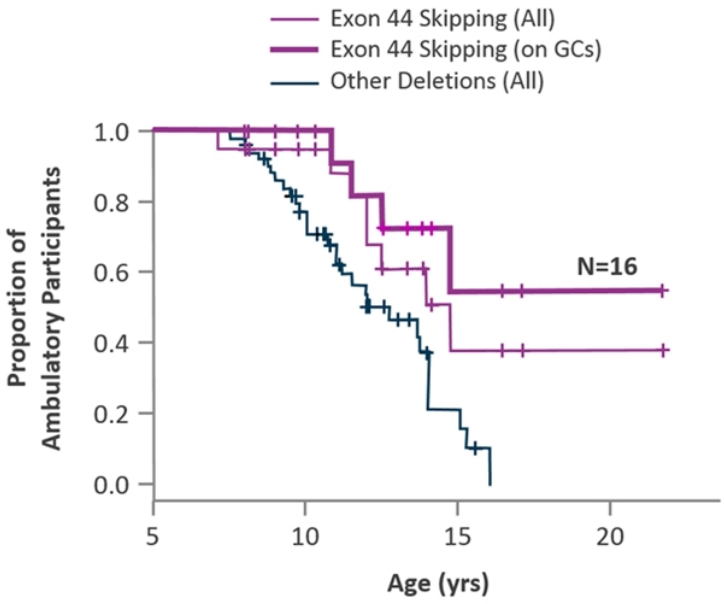


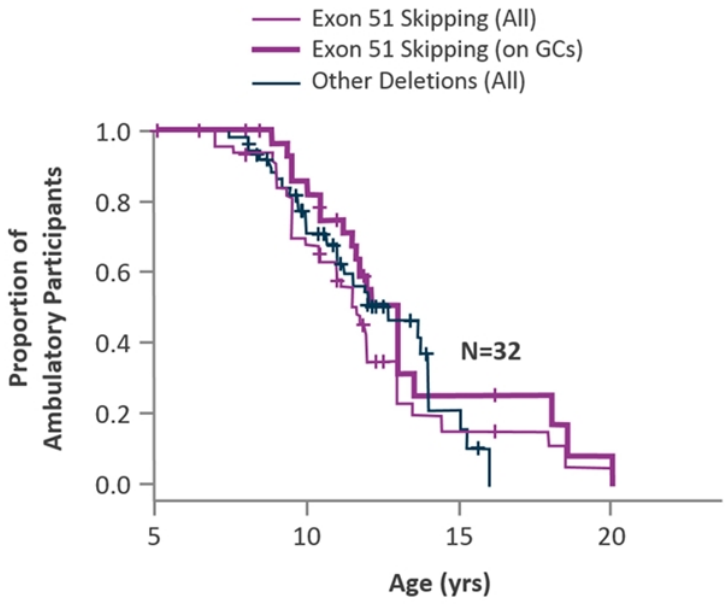
Study 201/202: 30 mg/kg and 50 mg/kg Resulted in Similar Dystrophin Response at Week 48



Absolute Values
Each bar graph N=6

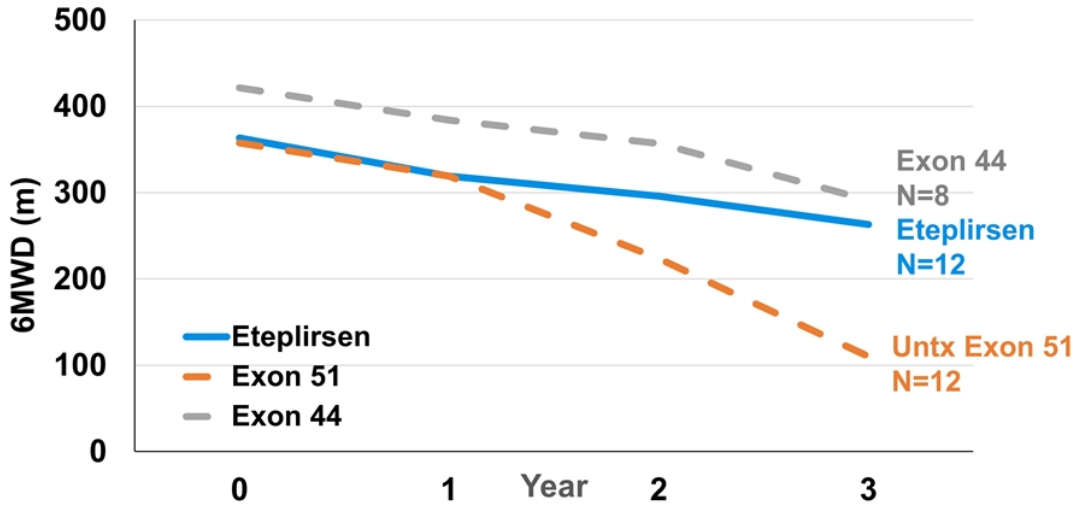
CINRG: Exon 44 vs Exon 51 Skip Amenable LoA



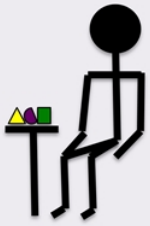


Bello et al. *Neurology.* 2016; 87:4(4)401-9

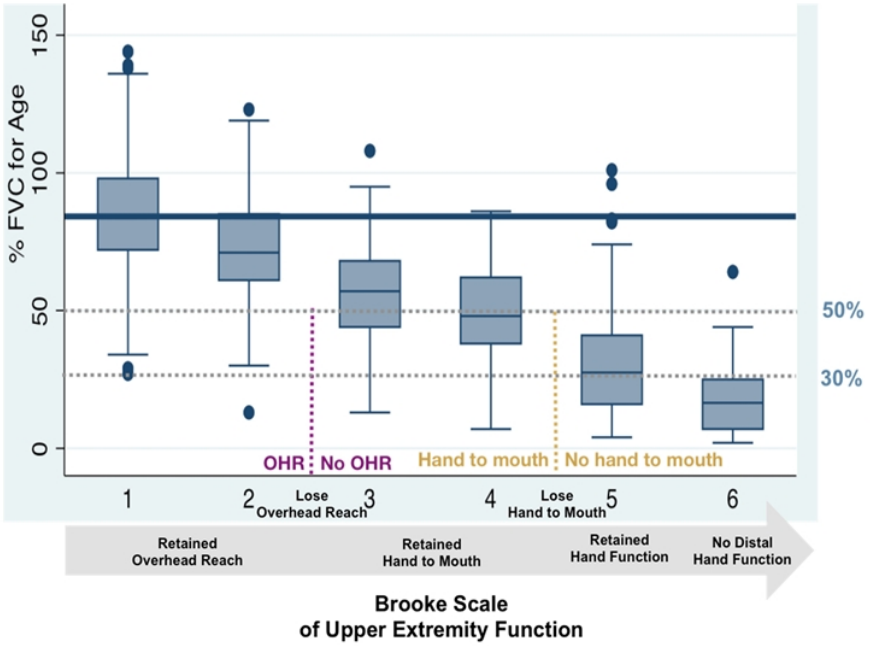
Longitudinal 6MWD:
Exon 44 Skip Amenable vs. Treated/Untreated Exon 51



Brooke Upper Extremity Functional Grade

1	2	3	4	5	6
					
Starting with arms at the sides, the patient can abduct the arms in a full circle until they touch above the head.	**Can raise arms above head** only be flexing the elbow or using accessory muscles.	**Cannot raise arms above head** but can raise an 8oz. glass of water to mouth.	**Can raise hands to mouth** but cannot raise an 8oz. glass of water to mouth.	**Cannot raise hands to mouth** but can use hands to hold a pen or pick up small objects from a table.	Cannot raise hands to mouth and has no functional use of hands.

FVC is Related to Upper Limb Function in DMD

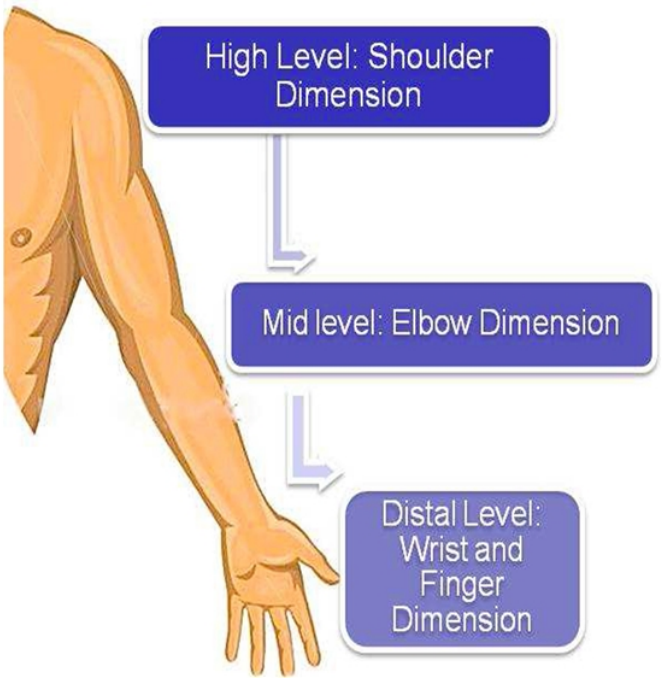


Patients with no hand function have very low FVC

CINRG Duchenne Natural History Study Data; n = 2311 observations. OHR = Overhead Reach

McDonald et al. Lancet 2017

PUL Assessment Items



- **1 PUL entry item to define the starting functional level (similar to Brooke score)**
- **4 items: HIGH LEVEL – SHOULDER DIMENSION**
- **9 items: MID LEVEL – ELBOW DIMENSION**
  includes timed items
- **8 items: DISTAL LEVEL – WRIST and HAND DIMENSION**

Study 4658-204
PUL Middle Domain – NON AMBULATORY

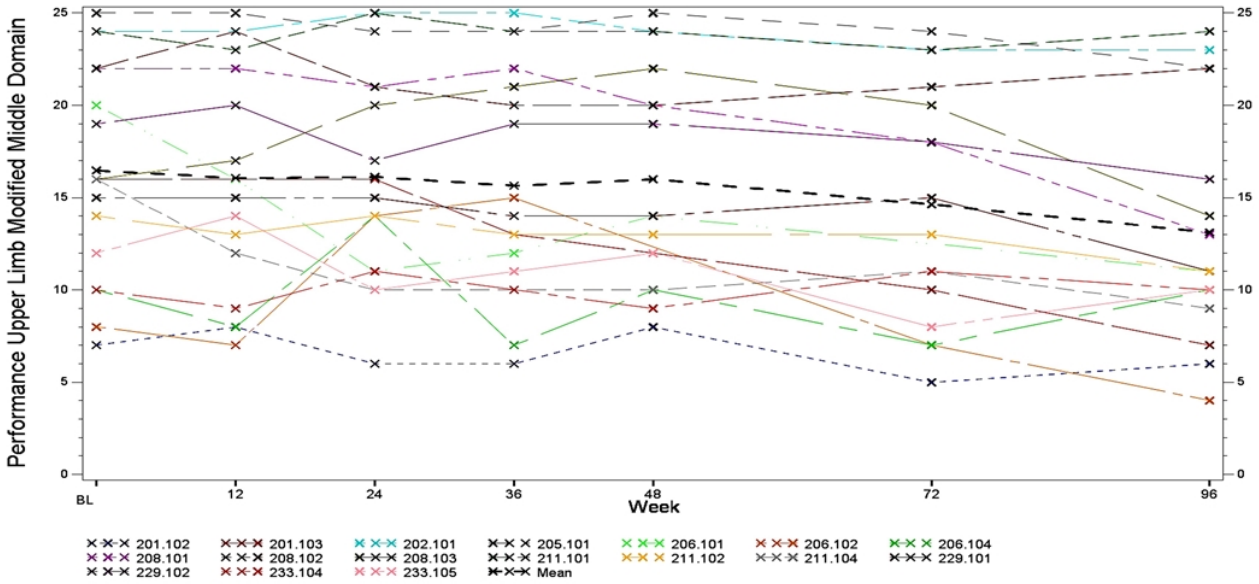


Figure 8: Day 180 JRAR Clinical