ATL1102 DATA PRESENTED AT MUSCULAR DYSTROPHY ASSOCIATION ANNUAL SCIENTIFIC CONFERENCE

Melbourne, Australia – 4 March 2024: Percheron Therapeutics Limited, an international biotechnology company focused on the development of novel therapies for rare diseases, is pleased to share three poster presentations released today at the Annual Clinical and Scientific Conference of the Muscular Dystrophy Association (MDA). The conference is being held in Orlando, FL, from 3 – 6 March 2024.

"These three presentations illustrate the breadth and depth of work ongoing with ATL1102 in muscular dystrophy," commented Percheron CEO, Dr James Garner. "We have gleaned an impressive body of data with the drug, and we are now focused on sharing it with researchers, clinicians, partners, and investors. Our presence at the MDA conference, one of the largest scientific meetings in the world for muscular dystrophy, is a valuable opportunity for us to raise awareness around the excellent work that the company and its collaborators have been doing."

Several Percheron personnel are attending the conference, and will be meeting with clinicians, researchers, and patient advocacy representatives over the course of the meeting.

Summary of Posters

A brief description of the posters follows, and the posters are appended to this announcement.

Poster V409 ATL1102 treatment of non-ambulant boys with DMD stabilizes function modifying plasma proteins with roles in immune, fibrosis, bone & growth physiology

This poster reports new data from the earlier phase IIa study of ATL1102 in Duchenne muscular dystrophy. As part of the study, blood samples were examined to measure the levels of key proteins associated with clinical parameters such as growth, bone density, and fibrosis. This work is an example of a scientific field known as *proteomics*, which assesses the effects of diseases and medicines through their impact on key proteins.

Many of the proteins measured showed favourable changes, implying that ATL1102 may provide clinical benefit in areas such as growth and bone density, in addition to the positive impact on upper limb function that has previously been reported.

Poster V410 **Mdx mice dosed with antisense to CD49d & dystrophin exon skip morpholino; improved muscle force & affected pathways support ATL1102 combination in DMD**

This poster is based on preclinical research exploring the combination of ATL1102 with an exon-skipping therapy, sometimes also referred to as a dystrophin restoration therapy. Since the experiment is performed in mice, a murine analogue of ATL1102 is used.

Both ATL1102 and the exon-skipping therapy improved muscle function. The combination of the ATL1102 analogue and the exon skipping therapy showed an improvement in the function of the exterior digitorum longus muscle which was generally more substantial than that seen with either drug alone. In addition, an analysis of gene expression in the muscle showed the combination of the two drugs affecting a wide variety of genes considered relevant to muscle function.

The company previously provided an overview of this work in July 2023[1], but this is the first time the research has been presented in detail at a scientific conference.

Poster M149 **Design of a Phase 2b study evaluating the efficacy and safety of ATL1102 in non-ambulant DMD**

The final poster provides an overview of the design of the ongoing phase IIb clinical trial of ATL1102 in non-ambulant boys with Duchenne muscular dystrophy (DMD).

As previously disclosed by the company, the study is a randomised controlled trial of two doses of ATL1102 versus placebo. The lower dose, 25mg, is the same as that reported in an earlier phase IIa study, which showed broad signals of efficacy in this population[2]. The primary endpoint of the study is the performance of the upper limb module (PUL2.0) at six months, and the study additionally evaluates a range of secondary endpoints. All patients then transition to an open-label extension phase in which they continue to receive ATL1102 at the originally allocated dose or, in the case of placebo patients, are re-randomised to receive ATL1102 at either of the two doses.

Recruitment to the trial is ongoing in Australia, the United Kingdom, Turkey, Bulgaria, and Serbia, and data is expected in 2H CY2024.

~ ENDS ~

[1] https://per.live.irmau.com/pdf/6c3c56e0-38a2-4326-bf90-982ddef2d817/Positive-new-DMD-Combination-Therapy-Data-in-mdx-mice.pdf
[2] IR Woodcock et al. (2004) *PLoS ONE* 19(1): e0294847

About Percheron Therapeutics Limited

Percheron Therapeutics Limited [ASX: PER | US OTC: ATHJY | FSE: AWY] is a publicly listed biotechnology company focused on the development and commercialisation of novel therapies for rare diseases. The company's lead program is ATL1102, an antisense oligonucleotide targeting the CD49d receptor. ATL1102 is currently the subject of an ongoing international phase IIb clinical trial for the treatment of non-ambulant patients with Duchenne Muscular Dystrophy (DMD), for which data is expected in 2H CY2024. The company previously reported promising results from an exploratory phase IIa study of in the same population and has been awarded orphan drug designation (ODD) and rare pediatric disease designation (RPDD) by the US FDA.

For more information, please contact info@PercheronTx.com.

This announcement has been authorized for release to the Australian Securities Exchange by the Board of Directors.

ATL1102 in Phase 2a in non-ambulant boys with DMD stabilizes function, modifying plasma proteins with roles in immune, fibrosis, bone and growth physiology

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BACKGROUND

- **Children with DMD** have dystrophin deficient muscles susceptible to contraction induced injury which triggers immune cells expressing CD49d that exacerbate muscle damage, fibrosis, loss of stem cells, and loss of function despite standard of care use of steroids[1]
- **ATL1102 is an immunomodulatory antisense drug to the CD49d** adhesion molecule on immune cells, and has completed a **successful Phase 2a trial** in 9 adolescent non-ambulant patients with DMD, modulating immune cells, and stabilizing function[2,3]
- **ATL1102 administered at 25mg once weekly** s.c for 24 weeks, on top of steroids in 8 of 9 patients, **was safe and further reduced white blood cells** (Table 1): CD3-CD49d+ NK cells (p=0.018 mixed model of repeat week 8,12 & 24 measures) and CD3+CD49d+ T cells at week 24 vs week 28 (p=0.010 paired T test*), 4 weeks post the end of dosing (EoD) [2,3]
- **ATL1102 stabilized multiple parameters** of disease progression, including performance of upper limb **function** (PUL2.0) [2,3], muscle **strength** (Myogrip, Myopinch) [2,3,4], versus losses reported in the literature, [3-6] and stabilized the **% fat in muscle MRI** compared to worsening when using corticosteroids. [4,5.]

Phase2a Mean and Median, Lymphocyte, T-cell CD49d and NK CD49d reductions

Phase 2a: White blood cell type changes (X10⁹ cells per litre)	Mean # and Change from baseline			Median % change from baseline	
	Baseline	24 weeks (EoD)	28 weeks	24 weeks (EoD)	28 weeks
Lymphocytes	3.68	-0.28	+0.19	-4.22%	+11.81%
CD3+ (CD4+ or CD8+) CD49d+ T cells	2.44	-0.28	+0.11*	-9.78%	+9.93%
CD3- (CD56+CD16+) CD49d+NK cells	0.45	-0.10	-0.10	-25. 9%	-7.28

Table 1. *Lymphocyte, T-cell CD49d+ and NK cell CD49d+ cell modulation at week 24 vs baseline and week 28*

Phase 2a PUL2.0, Myogrip, Myopinch, MRI Stabilization dosing ATL1102 for 6 months

PUL2.0 is a registration endpoint which measures muscle function in 3 dimensions:

ATL1102 patients demonstrated an overall improvement in PUL2.0 score* as well as other endpoints:

** 0.89 (SD 2.89) vs a loss in PUL2.0 of -2.00 (SD 3.018) in the matched external control group (n=20 patients, 39 6-month measurements) p=0.01.[3]*

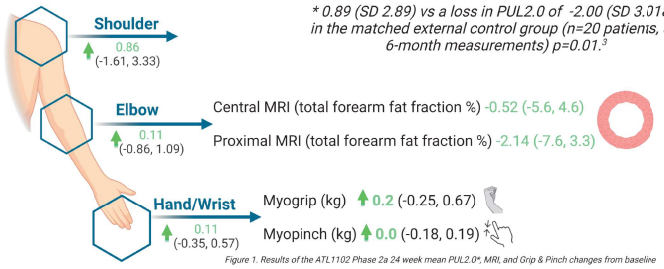
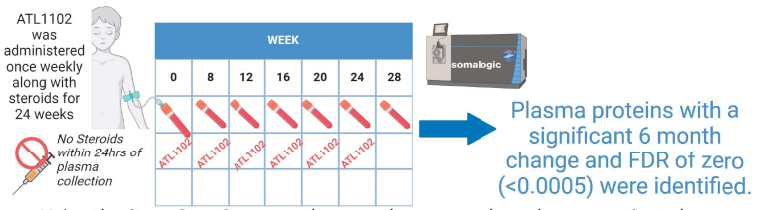


Figure 1. Results of the ATL1102 Phase 2a 24 week mean PUL2.0, MRI, and Grip & Pinch changes from baseline*

OBJECTIVES - STUDY OF PLASMA PROTEOMICS

1. To conduct a proteomics analysis of over 7000 plasma proteins from samples in the Phase 2 Study;
2. To compare changes to an external healthy subject control ;
3. Provide insights on the mode of action and broader biological activity of ATL1102 in DMD



Using the SomaScan® assay, a large scale, aptamer-based assay, patient plasma samples were tested and the normalized relative fluorescence units (nRFU) of over 7000 proteins was calculated.

RESULTS

- ATL1102 treated patients at 24 weeks had a statistically significant mean reduction of Thrombospondin-1 (-49%), and increases of LTBP4 (+20.7%), with roles in TGF beta mediated fibrosis, IGF-1 (+18.8%), & BMP6 (+34.4%) compared to baseline levels (FDR p-value <0.0005).
- Compared to healthy adult controls, nRFU, baseline median levels in the Phase 2a DMD study were near median for Thrombospondin-1 (THBS1) [6], and below median for LTBP4 & BMP6[6].

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DISCUSSION

ATL1102 modulates LTBP4, Thrombospondin-1, IGF-1 and BMP-6

Consistent mean changes during treatment were seen from week 8 to week 24 (shaded) for 3 proteins THSB1, IGF-I and BMP6, with LTBP4 increasing from week 12 to 24, all 4 proteins trending back towards baseline levels 4 weeks post dosing

- ATL1102 induced positive LTBP4 increases and THBS1 decreases in plasma, being 2 of 4 known DMD disease genetic modifier proteins with opposite effects on TGF- β1 involved in modifying the rate of loss of ambulation (LoA);[7,8] LTBP4 sequesters TGF-β1 and THBS1 activates Latent TGF- β1 involved in fibrosis
  - A recessive LTBP4 allele in 12% of patients with DMD, with greater levels of LTBP4, is associated with mild DMD providing 1-2 years delayed LoA[8].
  - A minor THBS1 allele with reduced expression appears protective against DMD progression[7].

- ATL1102 increases IGF-I levels, suggesting a potential for improving muscle and/or linear growth in DMD
- Baseline median IGF-I levels were 30481 nRFU vs adult controls 20463 nRFU (95%CI 10436-33178) and at week 24 37966 nRFU (95%CI 35334, 41405).

- ATL1102 induces increases to healthy control levels of BMP-6 and BMP-5[6], shown to play an important role in cartilage and bone formation[9,10] suggesting a potential for treating osteopenia in DMD[11]
  - Serum BMP6 levels are reportedly associated with improved elbow flexion in DMD patients and TGF- β.[12]

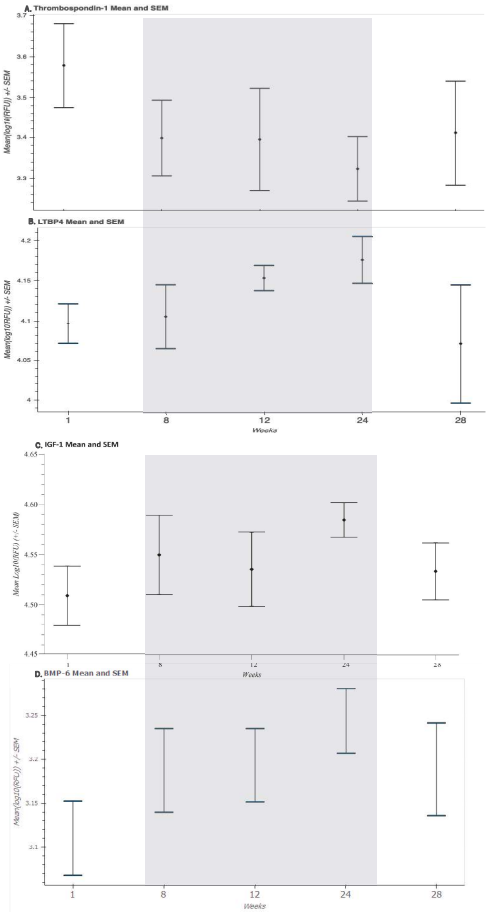
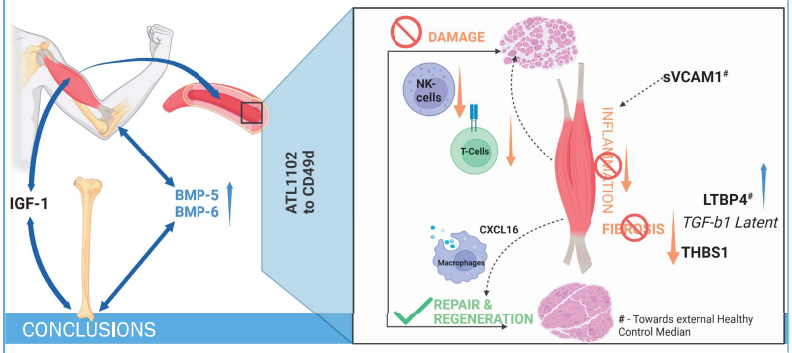


Figure 2a,b,c,d. *Mean (SEM) Results at baseline (1) to week 24 (end of dosing) changes and to w28 4 weeks past dosing*



CONCLUSIONS

- ATL1102 in non-ambulant patients reduces lymphocytes and modulates proteins (sVCAM-1[6]) with a role in CD49d inflammation and TGF-β1 fibrosis, and increases CXCL16[6] with a role in stem cell regeneration, stabilizing muscle function, strength and MRI muscle structure.
- ATL1102 modulates BMP-5 and 6, suggesting potential for improved bone density and IGF-1 suggesting a potential to increase muscle & linear growth, important given osteopenia[11] and growth retardation observed in DMD patients[13], exacerbated by steroids [12,14]

ATL1102 stabilization of multiple disease progression parameters together with the plasma protein effects position ATL1102 as an exciting prospect for the treatment of both non-ambulant and ambulant DMD patients

Muscular Dystrophy Association Annual Meeting
Orlando, FL March 2024

percheron THERAPEUTICS

In Collaboration with: murdoch children's research institute

Mdx mice dosed with antisense to CD49d & dystrophin exon skip morpholino; improved muscle force & affected pathways support ATL1102 combination in DMD subjects

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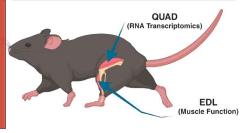
BACKGROUND

- **Boys with DMD** have dystrophin deficient muscles susceptible to injury which triggers the immune system, exacerbating muscle damage, and are currently treated with corticosteroids (CS) and Morpholino (PMO) Dystrophin exon-skipping restoration agents which produce little dystrophin (DESRA).[1]
- Current combination treatments are no more effective in maintaining ambulation than treatment with CS.[2]
- **ATL1102,** an antisense immunomodulatory drug to CD49d, has completed a successful Phase 2a trial in 9 adolescent non-ambulant DMD boys, stabilizing PUL2.0 muscle function and MRI lean tissue & fat mass better than CS .[3,4]
- **ASO to mouse CD49d RNA (ISIS348574)** equivalent to ATL1102 reduced CD49d mRNA expression in quadriceps and improved tibialis anterior skeletal muscle function providing protection from eccentric muscle damage. [4]

EXPERIMENTAL DESIGN

Mdx Mice (9 Weeks of Age)

- Mdx Saline
- MM oligonucleotide
- Exon Skip PMO*
- ASO to CD49d
- MM+PMO
- ASO+PMO



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Mdx Mice were treated with ASO to CD49d, negative control mismatch (MM) oligonucleotide, and Saline **once weekly for 8 weeks** or **PMO drug alone for 4 weeks**, and PMO in combination with either ASO or MM.
* PMO dystrophin pre-mRNA (+7–18; 5-GGCCAAACCTCGGCTTACCTGAAAT-3) exon 23 skipping restoration drug[5] was used at doses and for a period shown previously to increase dystrophin to similar low levels seen with DESRA in boys with DMD

EDL Functional Study

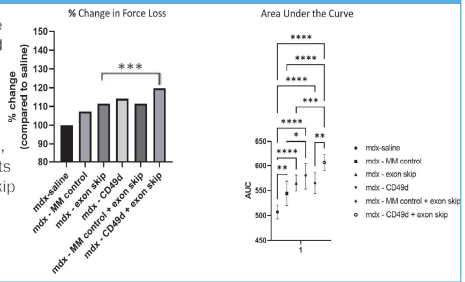
To assess EDL Function a series of 1 to 10 eccentric contractions were conducted, and the force measured expressed as a % of the force produced during the first (initial) contraction.

QUADRICEP RNA Seq and Pathway Analysis

RNA was isolated and RNA seq data generated and analyzed using R with quality control done using FastQC.
Normalization factors were calculated (TMM) and the Benjamini-Hochberg method Bayesian corrected False discovery rate (FDR) was determined to correct for multiple comparisons.[6]

RESULTS: EDL MUSCLE FUNCTION DATA

- After 1 lengthening contraction **only the combination dose** of ASO to CD49d and exon skip (PMO) improved EDL specific maximum force and eccentric muscle force compared to MM treated mice
- Post 10 lengthening contractions (Fig1), there were statistically significant effects with the combination above the exon skip PMO used alone *** (but not vs ASO), and differences with PMO vs Saline, & ASO to CD49d vs MM and Saline



DISCUSSION

- ASO to CD49d provides EDL functional benefits and changes Quadricep genes with beneficial roles in immune signaling ,reduced adiposity, improved muscle contractile function and satellite regeneration chemokines
- ASO to CD49d + PMO combination provides additional EDL functional benefits vs PMO (but not vs ASO) , and in the Quadricep modulation of additional pathways vs ASO and vs PMO affecting more immune response (macrophage pathways), new fibrosis, lipolysis (G0S2), IGF-I pathways, & creating more muscle (MYOM1)
- ATL1102 treated DMD patients in phase2a had some parallels to the ASO in mdx eg boys with MRI reduced muscle fat (mdx with lower FAPB4 in quad) & improved strength with higher CXCL16 in blood (mdx >EDL function & > quad CXCL16)

CONCLUSIONS

- Mdx mice treated with ASO to CD49d at weeks 9 to 16 demonstrated EDL muscle improvements;
- Greater EDL improvements with ASO+PMO combination therapy compared to saline and PMO monotherapy
- Quadricep gene expression modulated at week 16 by ASO reflects healthier immune, adipose, and stem cell muscle physiological outcomes;
- ASO+PMO combination showing additional immune, adipose outcomes and fibrosis and muscle improvement levels

This data supports the rationale for combination of ATL1102 (ASO to CD49d) with morpholino (PMO) dystrophin exon-skip replacement agents to improve therapeutic outcomes in boys with DMD

RESULTS: QUAD RNA SEQ

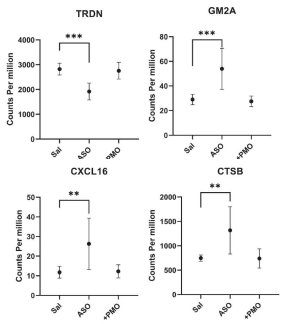
ASO monotherapy FDR <0.06 modulates 10 Targets

At FDR < 0.05, 2 genes were expressed significantly different unique to ASO monotherapy vs MDX-saline;

- Triadin ↓ 31% (calcium channel function)
- Gm2a ↑ 76% (immune neutrophil function)

At FDR < 0.06, there were 5 more genes unique to ASO

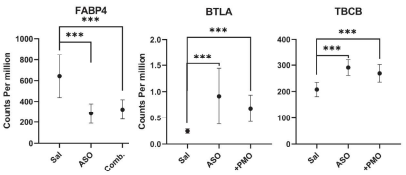
- CXCL16 (↑ 104%) a chemokine in satellite cell-myofibre regeneration[7],
- CSTB (↑ 64%) in neutrophil degranulation,
- PPP2R5E (↓ 48%) to increase glycogen metabolism ATP generation to help contraction,
- EIF3J2 (↓ 37%) in MHC class II antigen presentation
- Amd-Ps3 (↓ 68%) with unclear function.



ASO monotherapy FDR <0.06 + ASO+PMO FDR < 0.05

There were 3 targets shared between the ASO monotherapy and Combination ASO+PMO groups

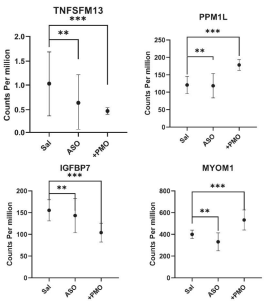
- FABP4 (↓ 55%, ↓ 49%) expressed in adipose suggests less fat[8],
- BTLA a low abundance lymphocyte[9] inhibitory receptor (up 18 fold, 11.8 fold),
- TBCB (↑ 37%, ↑ 27%) monocyte phagocytosis



ASO+PMO Combination Therapy FDR <0.05 (49 unique Targets)

At FDR < 0.05, 49 unique genes were found in the ASO+PMO vs the MM+PMO to FDR < 0.1.

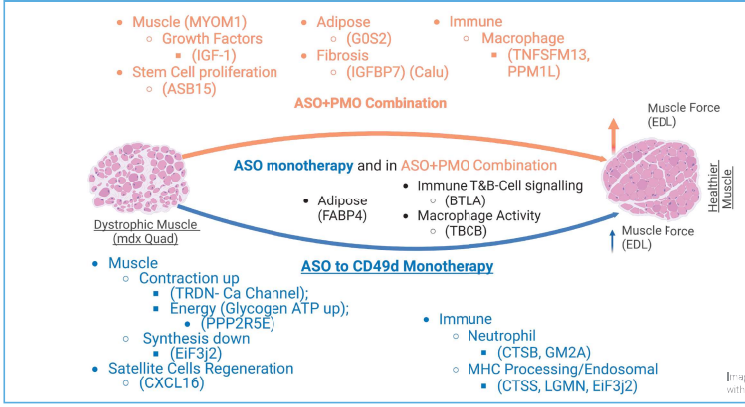
- Tnfsfm13 (↓ 91%) and PPM1L (↑ 50%) macrophage activity and immune signaling.[10]
- G0S2, a negative regulator of lipolysis, ↓ 61%.[11] for more lipolysis and less fat
- IGFBP-7 (↓ 34%) secreted from adipose-derived stem cells with a role in fibrosis and a modulator of IGF-I signaling as a growth-suppression factor[12,13]
- MYOM1 (↑ 33%),essential actor in muscle development, stability, and function suggesting more muscle [14]



RESULTS: GENETRAILS 3 PATHWAY ANALYSIS

- The 10 targets modulated by ASO to CD49d FDR <0.06 detected ≥2 genes in the following immune pathways:
  - Neutrophil degranulation- CTSB (+64%) , GM2a (+76%).
  - MHC class II antigen presentation- CTSB (+64%), TBCB^ (+37%), and Eif3J2
    - Additional MHC class II genes also identified at FDR <0.01 CTSS (+103%) & LGMN (+66%) (also in endosomal processing)
- The 49 targets modulated by ASO+PMO FDR <0.05 detected ≥2 genes in the following pathways:
  - Regulation of IGF transport and uptake ADAM10 (-46%) , CALU (-34%), IGFBP7(-34%)
  - Post-translational protein phosphorylation, ADAM10 (-46%) , CALU (-34%), IGFBP7(-34%)
  - Exercise induced circadian regulation G0S2 (-61%), KLF9 (+55%), STBD1 (-39%)

SUMMARY: RNA - PATHWAYS MODULTATED



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Muscular Dystrophy Association Annual Meeting
Orlando, FL March 2024

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In Collaboration with: murdoch childrens research institute

Design of a Phase 2b Study Evaluating the Efficacy and Safety of ATL1102, an Antisense Oligonucleotide to CD49d, in Non-Ambulant Patients with Duchenne Muscular Dystrophy

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BACKGROUND

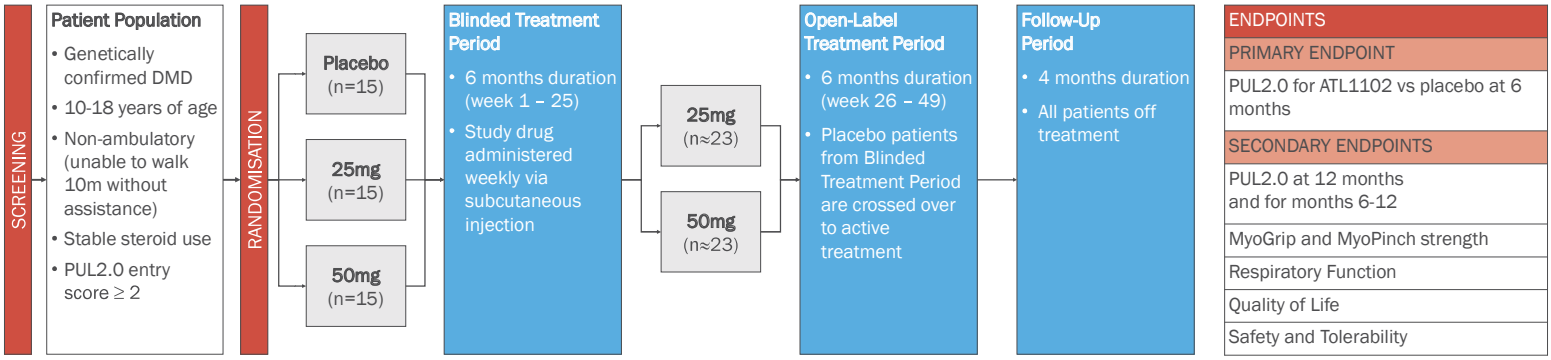
- **Duchenne muscular dystrophy** (DMD) is an X-linked recessive disorder caused by mutations in the dystrophin gene. A deficiency or absence of functional dystrophin, a structural protein in muscle, leads to progressive damage, chronic inflammation, and fatty infiltration of muscle tissues.[1]
- Critical to the inflammatory process associated with DMD is the trafficking of lymphocytes into muscle tissue. This is mediated by **CD49d**, a component of adhesion molecule VLA-4 (CD49d/CD29), on the lymphocyte surface, and by its ligands in the muscle tissue, including fibronectin and **osteopontin**.[2]
- **ATL1102** is an antisense oligonucleotide inhibitor of human CD49d. It has shown preclinical evidence of anti-inflammatory activity in a range of disease models and has previously been demonstrated activity in the clinic as a potential treatment for relapsing remitting multiple sclerosis.[3]

PRIOR CLINICAL DATA WITH ATL1102 IN DMD

- ATL1102 was previously investigated in a **phase IIa pilot study** in 9 non-ambulant boys with DMD.[4] Key efficacy signals were as follows:

Study Results (Efficacy) [at 6 months] (mean and 95% CI)		
Endpoint	**ATL1102 Result**	**Historical Comparator**
PUL2.0 function	↑ 0.9 (-1.33 – 3.11)	↓ 2.0 (-2.95 – -1.05)
MyoGrip strength (dominant hand)	↑ 0.2 kg (-0.25 – 0.67)	↓ 0.5 kg (-1.01 – 0.00)
MRI - total lean muscle area	↑ 13.9 mm^2 (-72.6 – 100.4)	↓ 32.1 mm^2 (-102.6 – 38.1)
Lymphocyte Counts	↓ 0.28 x 10^9 / L (-1.10 – 0.55)	↑ 0.47 x10^9 / L

STUDY DESIGN



ENDPOINTS
PRIMARY ENDPOINT
PUL2.0 for ATL1102 vs placebo at 6 months
SECONDARY ENDPOINTS
PUL2.0 at 12 months and for months 6-12
MyoGrip and MyoPinch strength
Respiratory Function
Quality of Life
Safety and Tolerability

STUDY DESIGN RATIONALE

Selection of Population

- **Non-ambulant patients** represent approximately half the DMD population and are least well served by existing therapies, with steroids being the standard of care.[2]

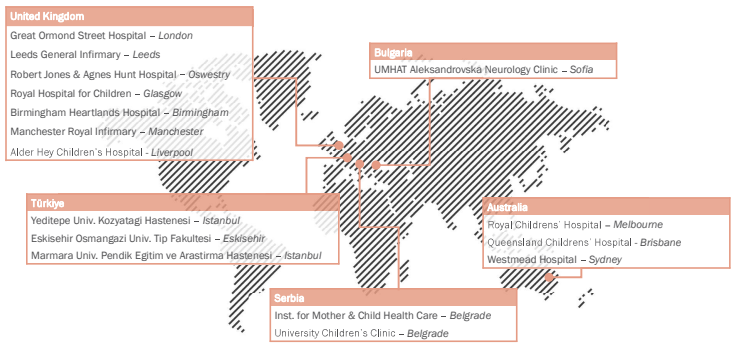
Selection of Primary Endpoint

- The **Performance of the Upper Limb** module (PUL2.0) was developed explicitly to quantify performance and disease progression in DMD patients, particularly in the non-ambulant setting.[5] It has been well validated and shown to be reliable and clinically meaningful.

Statistical Considerations

- A total of **15 subjects per arm** provides power of 80% and one-sided alpha of 0.05 to detect a difference between treatment and placebo of 2.7 points on the PUL2.0 scale, allowing for drop-outs.

CURRENT PARTICIPATING SITES



ClinicalTrials.gov NCT05938023

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