antisense THERAPEUTICS

(ASX: ANP)

Investor Presentation
July 2022

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Company Overview

- Biopharmaceutical company developing and commercialising antisense pharmaceuticals for large unmet markets with a focus in rare diseases (ASX:ANP | US OTC: ATHJY | FSE: AWY)
- Market Capitalisation A$54m
- Cash reserves at June'22 A$19.2m. No debt
- World-wide exclusive licenses to two drugs from **Ionis Pharmaceutical Inc** [(NASDAQ: IONS) Market cap US$5B]
- Lead program ATL1102 for Duchenne muscular dystrophy (DMD)
  - *Rare disease with high unmet medical need with no effective treatment for the more advanced sufferers*
  - *Successful Phase II clinical study and in advanced planning for next DMD clinical trial*
- Collaboration to study the neurological aspects of Long COVID-19 (Long Neuro COVID-19)
- Active research program to expand ATL1102's clinical application
  - *Rare muscle disease indication Limb Girdle Muscular Dystrophy R2 - positive animal results reported*
  - *Combination study with the dystrophin restoration drugs in mdx (DMD) mouse model*

Board and Management Team

Highly experienced Board and Management with prior success in drug development and commercialisation

MARK DIAMOND (Managing Director & CEO)

- 30+ years experience in the pharmaceutical and biotechnology industry.
- Previously employed in the US as Director, Project Planning/Business Development at Faulding Pharmaceuticals.
- Prior to this held the positions of Senior Manager, Business Development and In-licensing within Faulding's European operation based in the UK and International Business Development Manager with Faulding in Australia.

GEORGE TACHAS (Director of Drug Discovery & Patents)

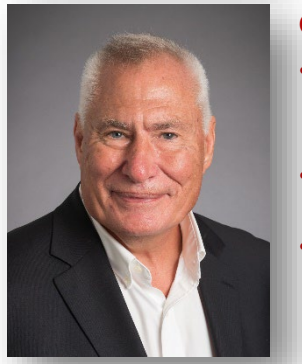
- Founding Director of Research of Antisense Therapeutics in 2001
- Immunologist and molecular biologist with a PhD from the University of Melbourne
- Diploma of Intellectual Property Law and has worked for two leading Australian patent firms
- Previously Head of Molecular Biology at the Cardiovascular Research Unit of Uni Melbourne's Anatomy Department.
- Inventor of using ATL1102 for the treatment of DMD.

NUKET DESEM (Director of Clinical & Regulatory Affairs)

- 25+ years' experience in global regulatory affairs, clinical development and project management obtained through her roles within the pharmaceutical/ biotechnology industry, including senior positions in various biotech companies.
- Previously employed at Antisense Therapeutics (2004–2010) as the Company's Development Director and responsible for the management of ANP's clinical trial programs.
- Major achievements included the successful conduct and completion of the Company's multinational Phase IIa clinical trial of ATL1102 for the treatment of Multiple Sclerosis.

CHARMAINE GITTLESON (Chair)

- Senior executive with international experience as a pharmaceutical physician and enterprise leader in pharmaceutical drug development, governance and risk management
- 15-year tenure with global biotechnology company CSL Limited
- Expertise in clinical research, medical safety, ethics for development, providing leadership across multiple therapeutic and rare disease areas.



GARY PACE (Non-Executive Director)

- 40+ years of experience in the development and commercialization of advanced tech in biotech., pharmaceuticals, and medical devices.
- Awarded a Centenary Medal by the Australian Government "for service to Australian society in research and development"
- In 2011 was awarded Director of the Year (corporate governance) by the San Diego Directors Forum.

GIL PRICE (Non-Executive Director)

- Clinical physician with a long-standing focus in drug development, adverse drug reactions, drug utilization and regulation.
- Experienced biotech executive and entrepreneur with a depth of expertise across clinical asset investment strategy, evaluation, financing and execution
- Previously was a non-executive director of Sarepta Therapeutics, Inc., where he helped guide Sarepta's transition to become a multi-billion dollar company with the first approved drug for DMD (sales approaching US$400M annually).

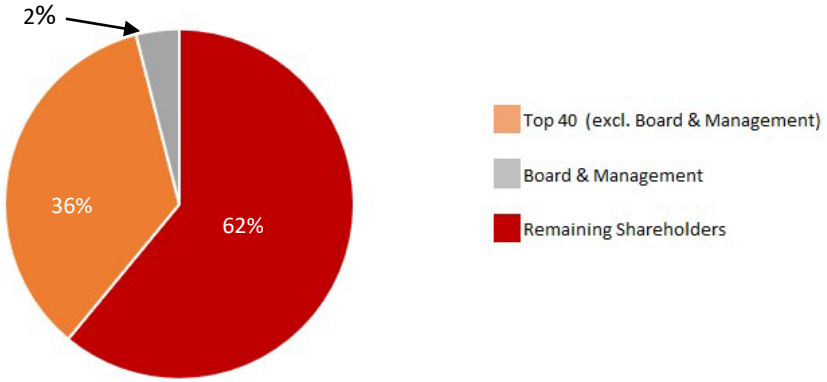
Antisense Corporate Overview

Share Price Performance

Company Details

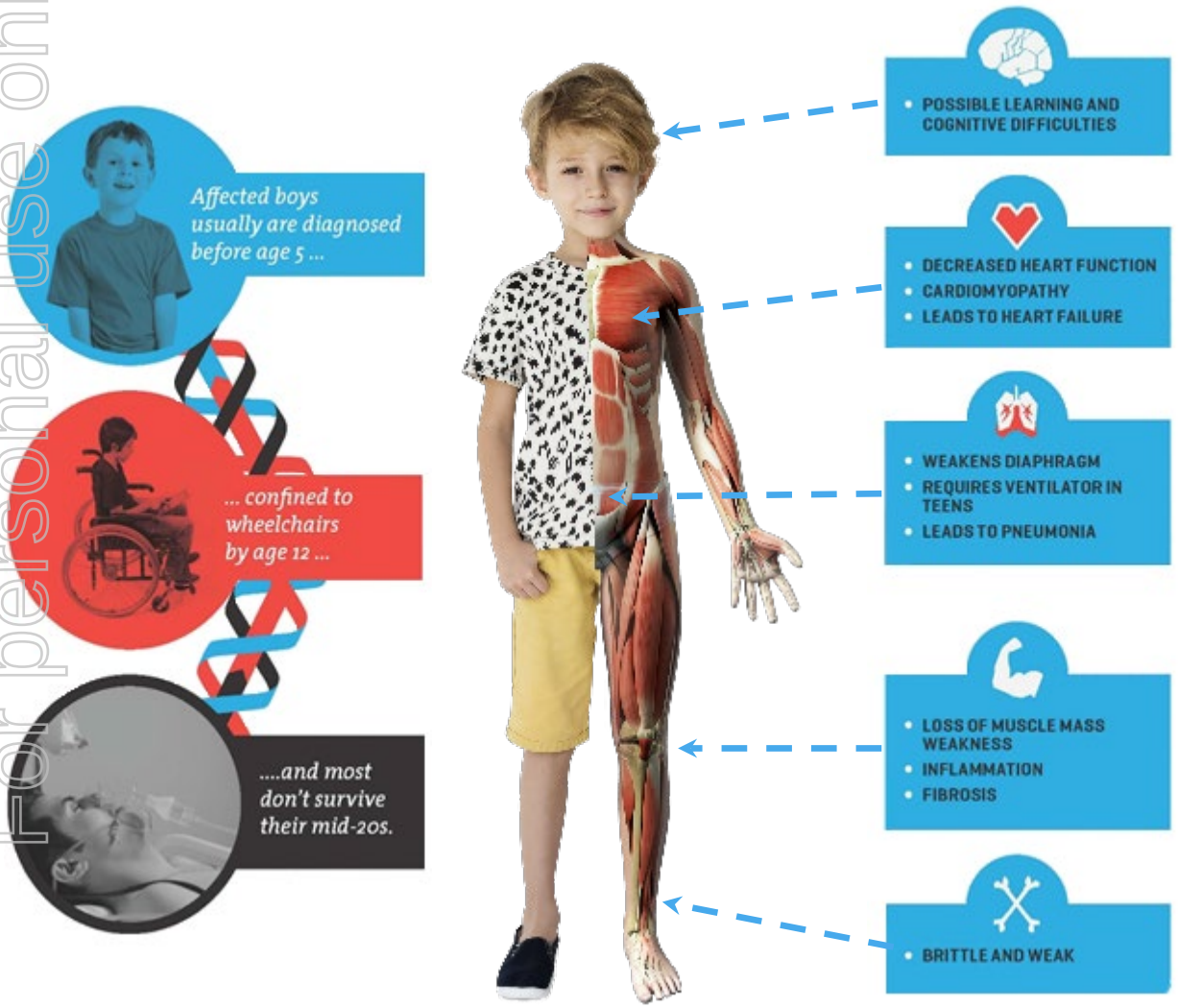
- Market Capitalisation @ $0.084 = $56M
- Ordinary fully paid shares on issue = 669M
- Cash at Jun'22 A$19.2m
- Largest shareholder
  - **Platinum Asset Management (5.23%)**
- Top 40 Shareholders
  - *Shares at 1 Jan '22 = 244M (36.5%)*
  - *Shares at 26 July '22 = 255M (38%)*

Current Register



What is DMD?

DMD is a rapidly progressing genetic disease resulting in low QOL and 100% mortality into patients' 30's



- Duchenne Muscular Dystrophy (DMD) is a devastating genetic muscular disease caused by loss of dystrophin with progressive muscle wasting & associated muscle injury leading to inflammation & fibrosis (100% mortality)
- Affects boys with an incidence of ~1 in 3,500 newborns[1] & prevalence of up to ~18,000 in US[1] and up to ~26,000 in EU[2]
- Key challenge in management of DMD patients is to reduce the inflammation and muscle fibre damage[3]
- Corticosteroids (CS) are the only therapy used to treat the inflammation in DMD[4] but have insufficient efficacy[5] & significant side effects including weight gain, reduced bone density & growth retardation[3]. CS not as effective in boys with a > number of T cells with high CD49d receptors[6].
- ATL1102 is designed to inhibit CD49d expression on lymphocytes and is being developed as a treatment to reduce inflammation in DMD.

Source of Image Cure Duchenne

[1] McNeil et al, Muscle Nerve, 2010, 41(6): p. 740-5
[2] http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/human/orphans/2015/05/human_orphan_001571.jsp&mid=WC0b01ac058001d12b
[3] Angelini and Peterle Acta Myologica 2012, XXXI: p. 9-15
[4] *Rosenberg et al,* Science Translational Medicine 2015, 7. p.299
[5] Miyatake et al Drug Design, Development & Therapy 2016, 10: p 2745–58
[6] Pinto-Mariz et al Skeletal Muscle 2015, 5: p. 45-55

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ATL1102 – Phase II Study Results

"Positive effects across multiple measures of muscle structure, function & strength"

Open label Phase II trial in nine non-ambulant (wheelchair bound) boys 10-18 years of age with DMD conducted over 24 weeks of dosing:

- Primary endpoint met with confirmation of drug's safety and tolerability
- Strong effects on secondary endpoints on activity markers and disease progression
  - Improvement or stabilisation across different measures of upper limb motor function (PUL.2.0) & strength (MyoGrip and MyoPinch)
  - Activity on the targeted CD49d immune cells consistent with drug's proposed mechanism of action
  - MRI data suggests stabilisation of percentage of fat in muscles and preservation of functional muscle mass
- International KOLs are supportive of Phase IIb plans

"The data certainly suggests an overall 'stabilisation' in disease progression at the very least which of itself is a very positive clinical outcome. MRI data confirms the positive changes at a muscular/cellular level and supports the observed physical stabilisation/ improvements in muscle strength and function.

The consistency of positive clinically relevant effects of ATL1102 treatment across muscle measures of structure, strength and function are very pleasing and provide great encouragement for the treatment of non-ambulant patients with DMD."

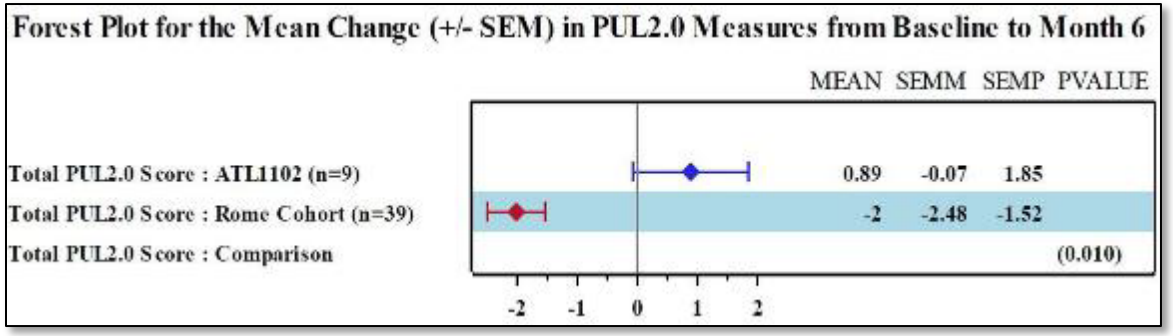
Professor Thomas Voit MD
Director, NIHR GOSH Biomedical Research Centre, UK

ATL1102 Phase II Study Data *(Continued…)*

Efficacy Parameters – Performance of Upper Limb Function (PUL.2.0)

- ATL1102 data presented at the 25th International Annual Congress of the World Muscle Society in 2020
- ATL1102 treated patients demonstrated a statistically significant improvement in the mean (SD) PUL2.0 scores for the 24 week treatment compared to external control (Rome Cohort)

ATL1102 Shows Statistically Significant Improvement vs Natural History Control in PUL 2.0 the registration endpoint for treatments in non-ambulant DMD



*"The level of improvement achieved **is very positive** and clinically relevant. As Total PUL2.0 is the key efficacy endpoint for seeking drug approval in non-ambulant patients with DMD, the comparative data further indicates **ATL1102's promising potential** to provide clinically meaningful benefits in the future treatment of non-ambulant DMD patients who have very limited treatment options."*

Professor Eugenio Mercuri,
Professor of Pediatric Neurology at the Catholic University, Rome, Italy

DMD Development Landscape

- *Limited emerging competition in non-ambulant space*
- *ATL1102 is a well-positioned differentiator from other products in development for treatment of DMD*

INTERVENTION MECHANISMS	ASSET	SPONSORS
INFLAMMATION & FIBROSIS	ATL1102	antisense THERAPEUTICS
	STEROIDS Emflaza, VBP15	*PTC, Santhera*
	ANTI-FIBROTICS pamrevlumab	*FibroGen*
CARDIAC & CALCIUM REGULATION	CARDIAC DRUGS Ifetroban	*Cumberland Pharma*
	CALCIUM BALANCE Rimeporide	*EspeRare Foundation*
DYSTROPHIN REPLACEMENT & RESTORATION	GENE THERAPY	*Pfizer/Sarepta*
	EXON 51 /EXON 53/EXON 45 SKIPPING	*Sarepta Therapeutics*
	Ataluren READTHROUGH THERAPY	*Nippon Shinyaku PTC Therapeutics*
RESPIRATORY CELL ENERGY	A0364	*Astellas Pharma*
MUSCLE GROWTH & REGENERATION	CAP-1002 (intravenous cell therapy)	*Capricor Inc*
	Givinostat	*Italfarmaco*

- **ATL1102 has a novel mechanism in reducing inflammation in DMD patients**
  - *Corticosteroids, dystrophin restoration agents and gene therapies being tested predominantly in ambulant patients*
  - *ATL1102's novel MoA in targeting CD49d suggests potential for drug to be used in combination with steroid*
  - *ATL1102 has potential to be synergistic with other products in development reducing competitive pressure*
  - *ATL1102 has an MoA that appears to be effective across genetic subtypes of DMD (a key differentiator among the exon skipping therapies) which increases the addressable patient pool*

Significant Opportunity (DMD and other markets)

ATL1102 - anti-inflammatory and immune modulating agent with potential for multiple clinical applications

ANTI-INFLAMATORY

The market^ size is expected global anti-inflammatory reach

US$191B** by **2027

(Fortune Business Insights)

^MS, Rheumatoid Arthritis, Asthma, Sinusitis Respiratory, IBD

DMD THERAPIES

The global DMD drug market estimated to reach

***US$4B** by **2023** and*

US$10B** by **2030

(Kamet Research)

CORTICOSTEROIDS

The global steroid market is forecast to attain value of

US$17 Billion** in **2025

(QY Research)

- Rare disease company **Sarepta Therapeutics Inc.** (NASDAQ: SRPT) (Mkt Cap **US$6B**) 2021 DMD Sales Revenue was > **US$700M**
- Cost of current DMD therapies: Deflazacort (Emflaza) - CS approved in US only - avg cost ~**US$93K**[1] per patient per year
  - Exondys 51 (exon-skipping/dystrophin restoration agent) avg cost in US ~**US$750K**[1] per patient per year
  - Ataluren (Translarna) - stop codon skipping/dystrophin restoration) cost in EU ~**US$320K**[1] per patient per year

Assuming:

$200K per annum with 50% of boys non ambulant at any one time

***ATL1102** has a **US$4B** market opportunity for DMD in US/EU*

[1] DelveInsight - Duchenne Muscular Dystrophy (DMD) Market Insight, Epidemiology, and Market Forecast—2030 (August 2021)

Note: Antisense's modelling is indicative and illustrative only and is not a forecast or projection of actual pricing of ATL1102 or the Company's ability to penetrate the depicted markets. A number of variable factors that will impact upon and influence actual pricing, market penetration and revenue and neither detailed and/or independent price modelling or audit has been undertaken.

ATL1102 for DMD Clinical Development

EU Phase IIb/III Clinical Trial

- Positive opinions received from EMA Paediatric Committee (PDCO) and Medicines and Healthcare products Regulatory Agency (MHRA) in the UK for ATL1102 Phase IIb/III Paediatric Investigational Plan (PIP)
    - *A multi-centre, randomised, double-blind placebo-controlled Phase IIb/III study of ATL1102 in non-ambulant patients dosed with ATL1102 for 12 months at two dose levels with a follow-on open label extension phase*
- Globally renowned Clinical Research Organisation (CRO) Parexel to conduct and manage the Phase IIb/III study
- Prof Thomas Voit MD (Director of NIHR GOSH UCL Biomedical Research Centre, UK) will be the Coordinating Principal Investigator
- First clinical trial application submitted in Germany (BfArM) for their evaluation and subsequent approval of the application
- Company is evaluating its clinical plans for ATL1102 in DMD and expects to provide a program update in the coming weeks

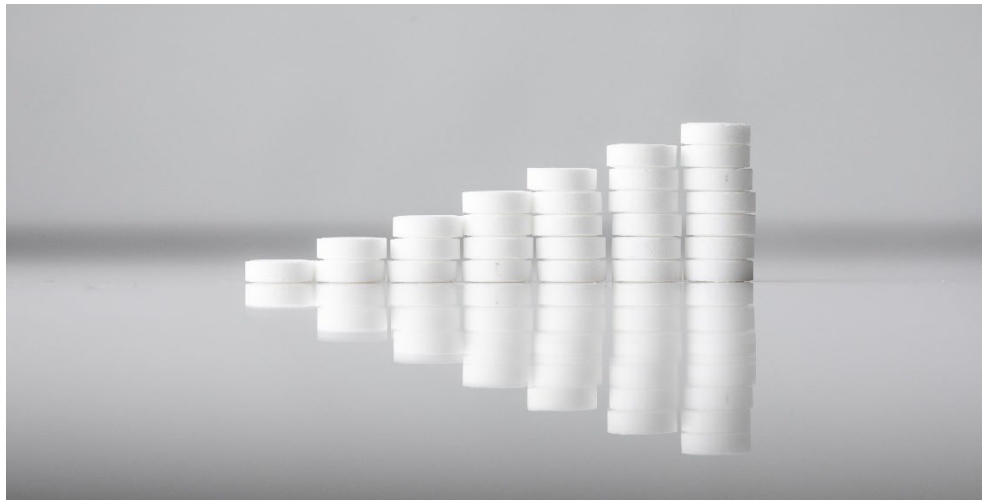
US Regulatory Plans

- FDA require a nine-month chronic monkey toxicology study of ATL1102 to support the dosing of patients beyond six months in US
- Feedback provided by FDA on the tox study protocol synopsis including FDA's concurrence with the proposed high dose
- Timing of the initiation of the tox study dependent on ATL1102 in DMD progress in Europe and continued interactions with the FDA
- Potential for rare pediatric disease priority review voucher (PRV) if FDA approval in the DMD indication before Sep 30, 2026. From 2017 - 2022 sales of PRVs ranged between US$80 - $150 million

ATL1102 New Indications

Expanding the clinical application of ATL1102

- Capitalising on the extensive data package generated to date to deepen the product pipeline
- Add further shareholder value and to diversify possible indication risk
- Leverage established core competencies (rare disease experience, scientific partnerships and collaborations)
- Commercially attractive space with limited competition, premium pricing, and ODD incentives
- Potential for ANP to move rapidly into the clinic based on positive animal data or out-license



ATL1102 New Indications *(Continued…)*

Key research collaborations

- Collaboration with Murdoch Children's Research Institute (MCRI) the largest child health research institute in Australia
  - *Study in another animal model of muscle disease: Limb Girdle Muscular Dystrophy R2* ***(positive data reported)***
  - *Combination study with the dystrophin restoration drugs in mdx (DMD) mouse model* ***(results anticipated 3Q/4Q2022)***
- Collaboration to study the neurological aspects of Long COVID-19 (Long Neuro COVID-19) with US based researchers led by Dr Igor Koralnik at the Northwestern Medicine Neuro-COVID clinic in Chicago
  - *Over 100 million people worldwide reported as having Long Covid – WebMD Nov 18, 2021*
  - *World first study to assess up to 7,000 plasma proteins in Long COVID-19 patients*
  - *Collaboration with a global leader in the clinical research of neurological aspects of Long COVID-19*
  - *Study to look for blood disease markers to assess if amenable to treatment including with ATL1102*
  - *Retained blood samples from patients tested in the US by leading proteomics group*
  - *Aim is to generate novel data to identify opportunities for the diagnosis, prognosis and treatment of Long Neuro COVID-19 and file for related IP protection*
  - *First results from testing anticipated* ***shortly***

murdoch children's research institute

Northwestern Medicine

ATL1102 in Limb Girdle Muscular Dystrophy R2

- LGMDR2, also referred to as dysferlinopathy, is a rare genetic muscle disease caused by mutations in the dysferlin gene (*DYSF*) that leads to significant reduction or absence of dysferlin protein levels in muscle fibers
- LGMDR2 is characterized by muscle inflammation, fibrosis, adiposity (fat) and progressive weakness in the hip and shoulder area (i.e. the limb girdle) proximal muscles with loss of ambulation and upper limb function in adulthood
- LGMDR2 affects ~ 1 in 125,000 people

No disease modifying agents in advanced development and no treatments shown to be beneficial in slowing the disease progression = significant potential commercial opportunity for ANP given unmet medical need

- Animal study conducted at MCRI in collaboration with Jain Foundation in the US
  - *Study results showed significant decreases in target CD49d RNA and key immune cell RNA levels in the muscle*
  - *Data supports progression into a longer-term efficacy study planned for **3Q/4Q2022** (pending the availability of suitably aged mice)*

With a singularly focused mission to find a cure for dysferlinopathy, we at the Jain Foundation are pleased to be working with Antisense Therapeutics and the MCRI on their research program evaluating the antisense to CD49d drug as a possible treatment of dysferlinopathy. We are encouraged by the results of the first study and look forward to the data of the longer dosing study to assess the efficacy of the treatment in mice in order to determine the potential of ATL1102 to be an effective therapy in this devastating and debilitating disease."

Laura Rufibach, Co-President of the Jain Foundation

Anticipated Key Events / Catalysts for 2022*

- ✓ Progress of European ATL1102 DMD Clinical trial: CTA submission, approval, trial initiation
- ✓ Publication of Phase II DMD clinical trial results in per reviewed scientific journal
- ✓ Presentations at scientific congresses
- ✓ Results of collaboration to study the neurological aspects of Long COVID-19 (Long Neuro COVID-19)
- ✓ Results of combination study with the dystrophin restoration drugs in mdx (DMD) mouse model
- ✓ Initiation/conduct of Limb Girdle Muscular Dystrophy R2 chronic animal study

* Indicative and not necessarily in anticipated chronological order

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