Neuromuscular Disorders Research Group
Biocruces Bizkaia Health Research Institute
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# Virginia Arechavala-Gomeza

## PERSONAL INFORMATION

Web site: www.arechavala-lab.com
Twitter account: @VArechavala
ORCID: http://orcid.org/0000-0001-7703-3255
Google Scholar: https://scholar.google.es/citations?hl=es&user=M4my6VQAAAAJ&view_op=list_works

## EDUCATION

2000-2004 **PhD Neurology** Institute of Psychiatry, **Kings College London**, UK
Thesis: "Molecular studies on mutant SOD1: the role of catalysis and aggregation in the pathogenesis of Amyotrophic Lateral Sclerosis". Submitted Sept 2004, Viva Dec 2004, **awarded 31 March 2005.**
1999-2000 **MSc Immunopharmacology** **University of Strathclyde**, Glasgow, UK
1993-1998 **Pharmacy Degree (5 years)** **University of the Basque Country**, Vitoria, Spain

## RESEARCH POSITIONS

Jan 2019-Current **Ikerbasque Research Professor**
June 2013- Current **Group Leader**
**Biocruces Bizkaia Health Research Institute**, Barakaldo, Spain
*Neuromuscular Disorders Group. Currently 4 researchers.*

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July 2008-May 2013 Research Associate Institute of Child Health, **University College London**, UK
*Preclinical development and **clinical trials** of first in man antisense therapy for DMD*

June 2005-June 2008 Research Associate Hammersmith Hospital, **Imperial College London**, UK
*Preclinical development and **clinical trials** of first in man antisense therapy for DMD*

Jan-May 2005 Research Associate
Sept 2000-Sept 2004 PhD Candidate Institute of Psychiatry, **Kings College London**, UK
*Research on Amyotrophic Lateral Sclerosis*

## FELLOWSHIPS AND AWARDS

2019 – current **Ikerbasque Research Professor**, Basque foundation for Science, Spain
2019 **Ezkertze Award,** Cruces University Hospital Excellence in Research award
2013 – 2021 **Miguel Servet Fellowship I and II**, Institute of Health Carlos III, Spain
2017 **Outstanding Mentor Award**, Society of Spanish Researchers in UK
2013 – 2017 **Marie-Skłodowska-Curie Fellowship**, Career Integration Grant, EU

## CITATION REPORT

Sum of publications: 38 (30 as main author), h-index 22, g-index=43 Sum of times cited: 3967. Average of citations per paper: 92, including highly cited first author publications in Lancet (1070), Lancet Neurology (844) and Human Gene Therapy (238) (Google Scholar, as of Sept 2022). For a detailed list, see PUBLICATIONS.

## NETWORKS

2018-2023 COST Action CA17103 (www.antisenserna.eu) Main proposer/Chair. Over 370 participants.
2013-2017. COST Action BM1207 (www.exonskipping.eu), Management Committee Member.
2007-current. Member, TREAT-NMD Network (www.treat-nmd.eu)
2005-2013 Postdoctoral researcher, MDEX consortium: "to promote collaborative translation research for Duchenne muscular dystrophy".

## CAREER BREAKS/PART TIME WORK

- April 2011- May 2013 Part-time work (80%, working 4 days per week)
- June 2010- April 2011 Maternity leave, 10 months
- Oct 2007- June 2008 Maternity leave, 9 months

## CLINICAL TRIALS

Accredited Good Clinical Practice (GPC) courses: 10th June 2008 and 9th October 2009.
Responsible of the selection and validation of the orphan drug **eteplirsen**/Exondys51.

Participated in the planning and coordinated the research part of these **two clinical trials**:
• Safety and Efficacy Study of Antisense Oligonucleotides in Duchenne Muscular Dystrophy. Clinicaltrials.gov identifier NCT00159250 (goo.gl/HMx7i)
• Dose-Ranging Study of AVI-4658 to Induce Dystrophin Expression in Selected Duchenne Muscular Dystrophy (DMD) Patients. Clinicaltrials.gov identifier NCT00844597 (goo.gl/vbHE8)

## TECHNOLOGY TRANSFER

Developed a screening method (myoblot) to test drugs for some neuromuscular disorders. Current collaborations based on that technology, including a contract with a drug re-purposing company (SOMBiotech, 25.000€, 2018).

## TEACHING ACTIVITIES

2019 Invited lecturer, seminar series, University of Cardiff, United Kingdom
2016-current Invited lecturer MSc Course "Master in Neurosciences" University of Barcelona, Spain
2018-Current Member, PhD program, "Molecular biology and Biomedicine" University of the Basque Country (UPV/EHU), Spain.
2014-current Invited lecturer MSc Course "Molecular biology and biomedicine" University of the Basque Country, Spain
Received teaching and supervising training at Imperial College and University College London (UCL)

## THESIS/VIVA TRIBUNALS

- S. Luna University of the Basque country (Reserve) 2022
- M. Sabater University of Valencia (Reserve President) 2022
- H. Goulle, The University of Western Australia, (External examiner). 2021
- X. Fernandez Universtitat de Barcelona (President of the tribunal) 2021
- J. Lasa, University of the Basque Country (Reserve) 2021
- A. Ballester, Universitat de Barcelona (President of the tribunal) 2020
- M. Christou, Cyprus School of Molecular Medicine. (External examiner) 2020
- J. Gonzalez, University of Santiago de Compostela, (Reserve President) 2019
- E. Cerro Herreros, University of Valencia, (President of the tribunal) 2018
- L. Lopez Gimenez, University of Cantabria, (Reserve), 2017
- G. Aldanondo Aristizabal, University of the Basque Country/EHU (Reserve) 2017
- I. Toral, University of the Basque Country/EHU (Reserve) 2016
- L. Gallego Villar, Universidad Autónoma de Madrid, (Member) 2015

## SUPERVISION OF STUDENTS AND POSTDOCTORAL FELLOWS

Dr Arechavala-Gomeza considers that is her role as a supervisor to encourage the development of the scientific careers of those under her direction. She also took part in a pilot mentoring scheme at UCL as a mentee and is now a mentor, encouraging young scientists into research careers. As part of several large research groups, she has informally supervised many students and research assistants, but only those officially reporting directly to her are detailed here.

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| 2022- 2023 | Ainhoa Benito, Degree student University of the Basque country. |
| 2022-current | Naomi Torres, Research Technician |
| 2022- current | Sergio Martín, Postdoctoral researcher |
| 2022 | Xabier Irusta, University of the Basque Country |
| 2019-2020 | Laura de la Puente, Master's, University of the Basque Country |
| 2018- current | Andrea López, Master's, PhD candidate, University of the Basque Country |
| 2017-2022 | Patricia Soblechero, PhD Candidate, Biocruces |
| 2019 | Karmele Alapont, Degree student, Autonomous University of Barcelona |
| 2019 | Aina Antón, Degree student, Universtitat de Barcelona |
| 2018- 2019 | Gabriela Gonzalez, Post-graduate Researcher, Biocruces |
| 2017-2018 | Irene Larrañaga, Master's student, University of the Basque Country |
| 2017-2019 | Edurne Albiasu, Research Technician, Biocruces |
| 2016- 2017 | Alba Paramio, Master's student, University of the Basque Country |
| March -2017 | Iker García Jimenez. Postdoctoral researcher, Biocruces |
| 2013- 2017 | Estibaliz Ruiz del Yerro, Research technician, Biocruces |

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| 2012 -2013 | Courtney Young, MRes in Biomedicine, University College London |
| 2011-2012 | Narinder Janghra, Research technician, University College London |
| 2012 | Ahmed Musawi, MSc in Cell and Gene Therapy, University College London |
| 2009-2010 | Rivka Steinberg, Research technician, University College London |
| 2009-2010 | Jihee Kim, Research technician and MSc student |
| 2006-2008 | Sofia Muses, PhD Student, Imperial College London |
| 2005-2008 | Carl Adkin, PhD Student, Imperial College London |

## PLANNING OF SCIENTIFIC MEETINGS

February 2023 DARTER final meeting, Bilbao
October 2022 DARTER Training school, Porto
July 2022 DARTER Scientific Matchmaking meeting, Barakaldo
October 2021 DARTER Retina meeting virtual
July 2021 DARTER COST Virtual Workshop 2021
May 2021 DARTER Online Training School "Communicating Research to Stakeholders"
February 2020 DARTER Training school "The guide to antisense development"
February 2019 Inaugural Meeting of COST Action Ca17103, >130 researchers. Local host
February 2018 Dystrophin quantification meeting. London, UK. Coordinator. >35 researchers
October 2016 21st International World Muscle Society Congress, Granada, Spain. >600 participants. Local organizing committee
February 2016 COST Workshop, BioCruces Health Research Institute 45 European participants. Local host.
November 2015 8th Biennial Congress of the Spanish Society for Gene and Cell Therapy, San Sebastian, Spain. 300 delegates. Local organizing committee and session host.
October 2013 9th International Myotonic Dystrophy Consortium (IDMC-9) meeting, >300 participants, San Sebastian, Basque Country, Spain in (http://goo.gl/S5au5). Scientific committee and session host.

## COMMISSIONS OF TRUST

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| 2021-2022 | NATA Delivery Research Challenge, Review Panel |
| 2021 | Navarra Biomed External evaluator |
| 2020-2022 | Member, Ethics committee for clinical research Basque Country. |
| 2020-2021 | Special Issue Editor Journal of Neuromuscular Diseases |
| 2019-2021 | Book Editor ¨"Antisense RNA Design, Delivery, and Analysis" Springer Science. |
| 2019-2020 | Special Issue Editor Genes, MDPI publisher |
| 2019-current | Grant Reviewer for the Muscular Dystrophy Campaign, UK |
| 2017 | Reviewer of SRUK/Bizkaia Talent, Young Basque Investigator Awards |
| 2016-current | Grant Reviewer for the French Muscular Dystrophy Association (AFM). |
| 2015-current | Member of the Scientific Committee, Society of Spanish Researchers in the UK. |
| 2013-current | Member of the European Medicines Agency's (EMA) experts list. |
| 2014-current | Grant Reviewer for the French National Research Agency (ANR). |
| 2013-current | Grant Reviewer for Action Duchenne Association (http://goo.gl/udyw5S). |

## SCIENTIFIC SOCIETIES

2018-current Oligonucleotide Therapeutics Society (www.oligotherapeutics.org)
2015-current Spanish Society for Gene and Cell Therapy (SETGyC)
2014-current. Marie Curie Alumni Association (MCAA) (www.mariecuriealumni.eu)
2014-current World Muscle Society (www.wms.com)
2014-current Society of scientist returning to Spain (CRE)
2012-current. *Founding Member*, Spanish Researchers in the United Kingdom (www.sruk.org.uk)

## PUBLIC ENGAGEMENT

Prof. Arechavala-Gomeza regularly collaborates with patient's associations, giving talks and seminars to patients and their families, translating scientific publications both into Spanish and into lay language (see http://www.arechavala-lab.com/publications/dissemination/), and participating in many fundraising activities (http://www.arechavala-lab.com/news/).

As a female scientist and a mother or two girls, the applicant is passionate about encouraging and inspiring students and particularly women to pursue a scientific career. In this role, she has collaborated with the Talentia (http://goo.gl/nxz9rO), Gioconda Salud and Jakin Mina programs with seminars to undergraduate students about scientific careers.

PUBLICATIONS

| Authors, title and date | Journal |
|---|---|
| Rodriguez-Outeiriño, et al "miR-106b is a novel target to promote muscle regeneration and restore satellite stem cell function in injured Duchenne dystrophic muscle" August 2022 | Molecular Therapy-Nucleic Acids |
| Soblechero-Martín et al "Lessons learned from developing an oligonucleotide drug for a rare disease" **April 2022** | Book Chapter RNA therapeutics, Elsevier |
| López-Martínez et a "Evaluation of exon skipping and dystrophin restoration in in vitro models of Duchenne muscular dystrophy" **February 2022** | Book Chapter Methods Mol Biol, Springer |
| Garanto and Virginia Arechavala "Antisense RNA therapeutics: A brief overview" **February 2022** | Book Chapter Methods Mol Biol Springer |
| Arechavala-Gomeza et al "Sharing" Negative" Results in Neuromuscular Research: A Positive Experience" **Sept 2021** | Journal of Neuromuscular Diseases |
| Soblechero-Martín et al. "Duchenne Muscular Dystrophy Cell Culture Models Created By CRISPR/Cas9 Gene Editing And Their Application To Drug Screening" **Sept 2021** | Scientific Reports |
| Soblechero-Martín et al "Utrophin modulator drugs as potential therapies for Duchenne and Becker muscular dystrophies" **May 2021** | Neuropath Appl Neurobiol |
| Hammond et al "Delivery of oligonucleotide-based therapeutics: challenges and opportunities" **April 2021** | EMBO Mol Medicine |
| Arechavala-Gomeza et al Special Issue "Genetic Advances in Neuromuscular Disorders: From Gene Identification to Gene Therapy" | Genes |
| Koehorst et al "The biomarker potential of miRNAs in myotonic dystrophy type I" **Dec 2020** | Journal of clinical medicine |
| Vasconcelos et al "Joining European Scientific Forces to Face Pandemics" **Dec 2020** | Trends in microbiology |
| López-Martínez et al. "An Overview of Alternative Splicing Defects Implicated in Myotonic Dystrophy Type I" **Sep 2020** | Genes |
| Echevarria et al. "Researcher's perceptions on publishing "negative" results and open access" **July 2020** | Nucleic Acids Therapeutics |
| Desviat et al. "EU-COST Actions: an effective tool for fostering international and national collaborative research for rare diseases" **Nov 2019** | Lancet Neurology |
| Aartsma-Rus et al "Report of a TREAT-NMD/World Duchenne Organisation Meeting on Dystrophin Quantification Methodology" **January 2019** | Journal of Neuromuscular Diseases |
| Hiller, M., et al., "A multicenter comparison of quantification methods for antisense oligonucleotide-induced DMD exon 51 skipping in Duchenne muscular dystrophy cell cultures". **October 2018** | PLoS One |
| Aartsma-Rus, A and **Arechavala-Gomeza V**. News and Views: "Why dystrophin quantification is key in the eteplirsen saga" **July 2018** | Nature Reviews Neurology |
| Ruiz-Del-Yerro, E, Mamchoui, K, Garcia-Jimenez I, **Arechavala-Gomeza**, V "Myoblots: dystrophin quantification by in-cell western assay for a streamlined development of DMD treatments" **August 2018** | Neuropath Appl Neurobiol |
| Godfrey, C Goyenvalle A, … **Arechavala-Gomeza** V *"Delivery is key: lessons learnt from developing splice switching antisense therapies"* **May 2017** | EMBO Molecular Medicine |
| Straub V, Balabanov P, Bushby K, Ensini M, Goemans N, De Luca A, Pereda A, Hemmings R, Campion G, Haas M, Kaye E, **Arechavala-Gomeza** et al *"Policy view: Cooperation among stakeholders to overcome challenges in orphan medicine development The example of Duchenne muscular dystrophy"* **July 2016** | Lancet Neurology |
| **Arechavala-Gomeza V*,** Khoo B*, Aartsma-Rus A* "Exon skipping therapy in the treatment of genetic diseases" ***Equal contribution. December 2014** | The Application of Clinical Genetics |
| Anthony K*, **Arechavala-Gomeza V*,** Taylor LE, et al. *"Dystrophin quantification: biological and translational research implications"* ***Equal contribution. Nov 2014** | Neurology |
| Anthony K, **Arechavala-Gomeza V**, Ricotti V et al "*Biochemical characterisation of patients with in- or out-of-frame DMD deletions pertinent to exon 44 or 45 skipping"* **Jan 2014** | JAMA Neurology (Archives of Neurology) |
| Anthony K, Feng L, **Arechavala-Gomeza** V, et al "*Exon Skipping Quantification by qRT-PCR in Duchenne Muscular Dystrophy Patients Treated with the* | Human Gene Therapy Methods |

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|---|---|
| *Antisense Oligomer Eteplirsen"* **Nov 2012** | |
| **Arechavala-Gomeza V,** Feng L, Morgan JE, et al. "*Antisense Measuring Dystrophin-faster is not necessarily better.*" CORRESPONDENCE. **August 2012** | Nature Reviews Neurology |
| **Arechavala-Gomeza V,** Anthony K, Morgan J, et al *"Antisense Oligonucleotide-Mediated Exon Skipping for Duchenne Muscular Dystrophy: Progress and Challenges."* **June 2012** | Current Gene Therapy |
| **Arechavala-Gomeza V,** S Cirak, K Anthony, et al "*Exon-skipping therapy for Duchenne muscular dystrophy - Authors' reply." CORRESPONDENCE.* **Jan. 2012** | Lancet. |
| Cirak S, Feng L, Anthony K, **Arechavala-Gomeza** V, et al *"Restoration of the Dystrophin-associated Glycoprotein Complex After Exon Skipping Therapy in Duchenne Muscular Dystrophy."* **Jan 2012** | Molecular Therapy |
| Anthony K, Cirak S, Torelli S, Tasca G, Feng L, **Arechavala-Gomeza** V, et al "*Dystrophin quantification and clinical correlations in Becker muscular dystrophy: implications for clinical trials."* **Nov 2011** | Brain |
| Cirak S*, **Arechavala-Gomeza*** V, Guglieri M, et al "*Exon skipping and dystrophin restoration in patients with Duchenne muscular dystrophy after systemic phosphorodiamidate morpholino oligomer treatment: an open-label, phase 2, dose-escalation study".* ***Equal contribution. August 2011** | Lancet |
| Malerba A, Sharp PS, Graham IR, **Arechavala-Gomeza** V, et al "*Chronic systemic therapy with low-dose morpholino oligomers ameliorates the pathology and normalizes locomotor behavior in mdx mice."* **Feb 2011** | Molecular Therapy |
| Kinali M, **Arechavala-Gomeza** V, Cirak S, et al "*Muscle histology vs MRI in Duchenne muscular dystrophy."***Jan 2011** | Neurology |
| **Arechavala-Gomeza V,** Kinali M, Feng L, et al *"Immunohistological intensity measurements as a tool to assess sarcolemma- associated protein expression."* **June 2010** | Neuropath Applied Neurobiology |
| **Arechavala-Gomeza V**, Kinali M, Feng L, et al "*Revertant fibres and dystrophin traces in Duchenne muscular dystrophy: implication for clinical trials."* **May 2010** | Neuromuscular Disorders |
| Popplewell LJ, Adkin C, **Arechavala-Gomeza** V, Aartsma-Rus A, et al "*Comparative analysis of antisense oligonucleotide sequences targeting exon 53 of the human DMD gene: Implications for future clinical trials."* **Feb 2010** | Neuromuscular Disorders |
| Meng J, Adkin CF, **Arechavala-Gomeza** V, et al. "*The contribution of human synovial stem cells to skeletal muscle regeneration"* **Jan 2010** | Neuromuscular Disorders. |
| Walmsley GL, **Arechavala-Gomeza** V, Fernandez-Fuente M, et al "*A Duchenne muscular dystrophy gene hot spot mutation in dystrophin-deficient cavalier king charles spaniels is amenable to exon 51 skipping."* **Jan 2010** | PLoS One |
| Kinali M*, **Arechavala-Gomeza*** V, Feng L, et al. *"Local restoration of dystrophin expression with the morpholino oligomer AVI-4658 in Duchenne muscular dystrophy: a single-blind, placebo-controlled, dose-escalation, proof-of-concept study".* ***Equal contribution. Oct 2009** | Lancet Neurology |
| **Arechavala-Gomeza V,** Graham IR, Popplewell LJ, et al "*Comparative analysis of antisense oligonucleotide sequences for targeted skipping of exon 51 during dystrophin pre-mRNA splicing in human muscle."* **Sept 2007** | Human Gene Therapy |
| CE Shaw, **V Arechavala-Gomeza**, A Al-Chalabi. *"Familial amyotrophic lateral sclerosis"* **Dec 2006.** BOOK CHAPTER | Handbook of Clinical Neurology |

## SELECTED MEETINGS

### Selected oral presentations

- The Castle Delivery Meeting Redux2. Delivery of Nucleic Acid Therapeutics: Biology, Engineering and Development. 2023. Siracusa, Sicily. Invited speaker
- Summer course on advanced therapies June 2022, San Sebastian, Invited speaker and moderator
- European Society of Human Genetics (ESHG) Conference 2022, June 2022, Vienna. Invited speaker.
- 8th international therapeutic nucleic acids conference, Oligo 2022 April 2022, Oxford Invited Speaker

- Meeting of the German Society for Experimental and Clinical Pharmacology and Toxicology (DGPT), March 2022. Invited speaker
- Academia de Ciencias médicas de Bilbao" 5th October 2021. Invited speaker
- Bio Türkiye 11 Sept 2021 (online) "Delivery is key: lessons from developing splice switching oligonucleotides"
- Duchenne Parent project Spain Workshop (online) "Compartir resultados "negativos" puede ser positivo" 7 Sept 2021
- DARTER Online Workshop "Open Access and "negative" results" July 2021
- DARTER Training school "Unexpected but still valuable: why and how to communicate "negative results" June 2021
- ICNMD 2021 "Dystrophin Quantification in Diagnostic Pathology and Clinical Trials: Implication for Diagnosis and Translational Research" Valencia (virtual) May 2021
- Spanish cardiology society "Terapias avanzadas para enfermedades neuromusculares", Málaga (virtual) Feb 2021
- "Developing treatments for rare diseases: much more than finding a new drug" CIC-Biogune (online webinar) July 2020
- "Developing treatments for rare diseases: much more than finding a new drug" IBIS Sevilla March 2020
- Asociación Enfermos Neuromusculares de Bizkaia (BENE) "Avances y retos de la investigación" Terapia "genica" en DMD" Bilbao, Spain. Dec 2019
- CORBI summit Encuentro de mujeres científicas. Lugo Dec 2019
- Second Spanish Meeting on Oligonucleotide Therapeutics (SMOT2). "Delivery hurdles in the development of therapeutic oligonucleotides" INCLIVA, Valencia, Nov 2019
- Reunión de primavera Neuromusculares, Sociedad Española Neurología. Bilbao, Mayo 2019.
- XXXII Congreso nacional de Enfermedades Neuromusculares, Federación ASEM, Las Palmas, Mayo 2019
- Jornada Cardiogenética Murcia, Nov 2018
- First Spanish Meeting on Oligonucleotide Therapeutics (SMOT1). "Preclinical and clinical studies using oligonucleotide therapies in Duchenne" University of Valencia, June 2018 https://www.incliva.es/site/files/adjunto/formacion/first-meeting-smot1-fv2.pdf
- I Congreso nacional sobre la distrofia muscular de Duchenne y Becker "Edición génica de células de modelos animales para un tránsito más rápido de la terapia a la clínica" Madrid, 26 May 2018
- Webinar "Edición génica CRISPR/Case n las distrofias musculares de Duchenne y Becker" Duchenne Parent Project Spain, 8 Mayo 2018 https://www.youtube.com/watch?v=cvR4zRmTlpU
- VII Jornada científica, Federación de Enfermedades Raras FEDER, "Desarrollo de terapias para enfermedades raras" Bilbao, 18 Marzo 2018
- Jakinmina "Developing treatments for rare diseases: much more than finding a new drug" Jakiunde, Academy of Sciences, Arts and Leters, Deusto Business School, 26 Jan 2018 https://www.jakiunde.eus/actividades/jakin-mina/d/e/jakin-mina-2017-2018-bizkaia/
- XXXI Congreso nacional de Enfermedades Neuromusculares, Federación ASEM, Granada, November 2017 https://www.youtube.com/watch?v=IR9DLzPdPhM&t=1886s
- Science plus, ERA Career day, Bizkaia Aretoa, Bilbao September 2017. https://www.youtube.com/watch?v=pB6HbYt-TNQ
- II Research Workshop Duchenne Parent Project Spain, "Research of the Neuromuscular Disorders Group at IIS BioCruces" Barcelona, Spain. September 2017
- "Nuevas terapias en distrofia muscular de Duchenne", Pediatrics Service, Cruces University Hospital, Barakaldo, Spain June 2017
- Symposium "Todos somos raros": "QUADRES: QUAntification of Dystrophin Restoration by Exon Skipping", Spanish ministry of Health, Madrid, June 2017
- "Investigación en Distrofia muscular de Duchenne" Manzana solidaria, colegio Europa, Getxo 10 Mayo 2017.
- "Estrategia topagunea: "Atraer, retener y vincular el talendo en Bizkaia" Estrategia empresarial y diputación de Bizkaia, BIC Barakaldo April 2017
- Neurokafe, "Orphan drugs, rare diseases: a coffee break story" BioCruces Heatlh Research Institute, Barakaldo, Spain February 2017
- RefBio Pyrenees Biomedical Network Elevator pitch "Quantification of target proteins in cell culture: how to accelerate the transit from the bench to the clinic". Toulouse, January 2017
- Asociación Enfermos Neuromusculares de Bizkaia (BENE) "Avances y retos de la investigación" Bilbao, Spain. Diciembre 2016

- Reunión extraordinaria del grupo GEVANENEM: "Actualización en distrofinopatías y casos clínicos". "Distrofinopatías: estado actual de las terapias basadas en salto del exón" San Sebastián November 2016
- Action Duchenne International Conference. "World Muscle Society Conference highlights" London, United Kingdom. November 2016
- Asociación Ayuda enfermedades raras solidaridad y esperanza Gipuzkoa "Obstáculos en el desarrollo de terapias para enfermedades raras" Azpeitia, Spain, October 2016
- Duchenne Parent Project Spain Workshop, Barcelona, Spain. September 2016
- Summer course, University of the Basque Country, San Sebastián, Spain. June 2016
- 8th Biennial Congress of the Spanish Society for Gene and Cell Therapy, San Sebastián, Spain. Nov 2015
- Symposium of the Catalan Neuromuscular Association (ASEM Catalunya). June 2015
- Galdakao Hospital, Spain. Invited speaker. May 2015
- Horizon 2020 Infoday: Basque success stories January 2014
- Congress of the Spanish Neurology Society, Barcelona, Spain. November 2013
- International Symposium Ramon Areces Foundation, Madrid, Spain "Dystrophinopathies. Current concepts over antisense therapeutic strategies. Clinical Trials." (http://goo.gl/Fafbn) November 2012
- XXVI Congress of the Spanish Human Genetics Association, Spain "RNA Therapy in Duchenne muscular dystrophy: exon skipping" April 2011
- 7th Christmas Meeting, Neurosciences Institute, Alicante, Spain. "Exon skipping with antisense oligonucleotides: Restoration of dystrophin expression in Duchenne muscular dystrophy" December 2010
- Hospital de Cruces, Bizkaia, Spain "RNA therapy in Duchenne muscular dystrophy" December 2010
- Parent Project conference, Rome, Italy. "Restoration of Dystrophin Expression in DMD Using Morpholino Oligomer AVI-4658" February 2010
- London Myology forum, UK. "Local restoration of dystrophin expression in Duchenne Muscular Dystrophy"November 2009
- 14th International Congress of the World Muscle Society, Geneva, Switzerland. "Restoration of Dystrophin Expression in Duchenne Muscular Dystrophy: A Single Blind, Placebo-Controlled Dose Escalation Study Using Morpholino Oligomer AVI-4658" Neuromuscular Disorders September 2009 (Vol. 19, Issue 8, Page 659).

Selected poster communications

| Year | Author | Meeting | Published abstract | City, Country |
|---|---|---|---|---|
| 2022 | López-Martínez; Soblechero-Martín; Catalli, Jauregi-Barrutia,; Nogales-Gadea; Kapetanovic-Garcia; Arechavala-Gomeza | International Congress of the World Muscle Society | Neuromuscular Disorders | Halifax, Canada (online) |
| 2022 | López-Martínez; Soblechero-Martín; Martín-González; Catalli, Jauregi-Barrutia,; Nogales-Gadea; Kapetanovic-Garcia; Arechavala-Gomeza | Myology | | Nice, France |
| | López-Martínez; Soblechero-Martín; Catalli, Jauregi-Barrutia,; Nogales-Gadea; Kapetanovic-Garcia; Arechavala-Gomeza | Neurogune | | Pamplona, Spain |
| 2022 | López-Martínez; Soblechero-Martín; Catalli Nogales-Gadea; Kapetanovic-Garcia; Arechavala-Gomeza | International Congress Neuromuscular Diseases (ICNMD 22) | Journal: Journal of Neuromuscular Diseases, vol. 9, no. s1, pp. S1-S331, 2022 10.3233/JND-229001 | Brussels, Belgium |
| 2022 | A López-Martínez, P Soblechero-Martín, C Catalli, A Jauregui-Barrutia, S Kapetanovic-Garcia, V Arechavala-Gomeza | 13th International Myotonic Dystrophy Consortium Meeting | | Japan (online) |

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| 2021 | P. Soblechero-Martín, A. López-Martinez, V. Arechavala-Gomeza | International Congress of the World Muscle Society | Neuromuscular Disorders https://doi.org/10.1016/j.nmd.2021.07.186 | Online |
| 2021 | P. Soblechero-Martín, A. López-Martinez, V. Arechavala-Gomeza | International Congress Neuromuscular Diseases (ICNMD 21) | Journal of Neuromuscular Diseases, vol. 8, no. s1, 10.3233/JND-219006 | Valencia (online) |
| 2019 | P. Soblechero-Martín, E. Albiasu-Arteta, J. Poyatos-García, G. González-Iglesias, A. Anton-Martinez, A. López-Martinez, V. Arechavala-Gomeza | European Society for Gene and Cell Therapy | Human Gene Therapy http://doi.org/10.1089/hum.2019.29095.abstracts | Barcelona, Spain |
| 2019 | J. Poyatos-García, P. Soblechero-Martín, E. Albiasu-Arteta, G. González-Iglesias, A. Anton-Martinez, A. López-Martinez, V. Arechavala-Gomeza, RP Vázquez-Manrique, P Martí, N Muelas, C Gomis, JJ Vílchez | International Congress of the World Muscle Society | Neuromuscular Disorders https://www.nmd-journal.com/article/S0960-8966(19)30792-8/abstract | Copenhagen, Denmark |
| 2019 | P. Soblechero-Martín, E. Albiasu-Arteta, J. Poyatos-García, G. González-Iglesias, A. Anton-Martinez, A. López-Martinez, V. Arechavala-Gomeza | International Congress of the World Muscle Society | Neuromuscular Disorders https://doi.org/10.1016/j.nmd.2019.06.405 | Copenhagen, Denmark |
| 2019 | P Soblechero-Martin, I Larrañaga, G. Gonzalez, E Albiasu-Arteta, V Arechavala-Gomeza | International Congress on Myology | | Bordeaux, France |
| 2018 | E Ruiz-Del-Yerro, P Soblechero-Martin, I Larrañaga, E Albiasu-Arteta, V Arechavala-Gomeza | Neurogune | | Vitoria, Spain |
| 2018 | P Soblechero-Martin, I García-Jiménez, E Ruiz-Del-Yerro, E Albiasu-Arteta, V Arechavala-Gomeza | Neurogune | | Vitoria, Spain |
| 2018 | E Ruiz-Del-Yerro, P Soblechero-Martin, I Larrañaga, E Albiasu-Arteta, V Arechavala-Gomeza | International Congress on Neuromuscular Disorders. | Journal of Neuromuscular Disorders, vol. 5, no. s1, pp. S1-S408, 2018 https://doi.org/10.3233/JND-189001 | Vienna, Austria. |
| 2018 | P Soblechero-Martin, I García-Jiménez, E Ruiz-Del-Yerro, E Albiasu-Arteta, V Arechavala-Gomeza | International Congress on Neuromuscular Disorders. | Journal of Neuromuscular Disorders, vol. 5, no. s1, pp. S1-S408, 2018 https://doi.org/10.3233/JND-189001 | Vienna, Austria. |
| 2017 | P. Soblechero-Martín, E. Ruiz-Del-Yerro, I. García-Jimenez, V. Arechavala-Gomeza. | Science + ERA Career Day, | | Bilbao, Spain |

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| 2016 | I García, E Ruiz-Del-Yerro, V Arechavala-Gomeza | International Congress of the World Muscle Society | Neuromuscular Disorders, Vol. 26, S159–S160 | Granada, Spain |
| 2016 | E Ruiz-Del-Yerro, I García, V Arechavala-Gomeza | International Congress of the World Muscle Society | Neuromuscular Disorders, Vol. 26, S159 | Granada, Spain |
| 2016 | E Ruiz-Del-Yerro, I García, V Arechavala-Gomeza | Neurogune, | | Bilbao, Spain |
| 2015 | M. Hiller, … Aartsma-Rus, A. Ferlini, V. Arechavala-Gomeza, N. Datson, P. Spitali | 11th Annual Meeting Oligonucleotide Therapeutics Society, | | Leiden, Netherlands |
| 2015 | E Ruiz-Del-Yerro, I García, V Arechavala-Gomeza | International Congress of the World Muscle Society | Neuromuscular disorders October 2015, Volume 25, Supplement 2, , Page S254 | Brighton, UK |
| 2015 | E Ruiz-Del-Yerro, I García, V Arechavala-Gomeza | Science + Meeting | | Bilbao, Spain |
| 2014 | S. Torelli, K Anthony, V Arechavala-Gomeza, et al | UK Neuromuscular Translational Research Conference 2014 | Neuromuscular disorders (March 2014 Volume 24, Supplement 1, Page S11) | London, UK |
| 2013 | V Arechavala-Gomeza, C Godfrey, A Hibbert et al | UK Neuromuscular Translational Research Conference 2013 | | Oxford, UK |
| 2012 | K Anthony, V Arechavala-Gomeza et al. | 17th International Congress of the World Muscle Society | Neuromuscular Disorders (Vol. 22, Pages 804–908) | Perth, Australia |
| | L Popplewell, V Arechavala-Gomeza, et al<br>K Anthony, S Cirak, S Torelli, G Tasca, L Feng, V Arechavala-Gomeza, et al | UK Neuromuscular Translational Research Conference 2012 | Two abstracts: Neuromuscular Disorders (Vol 22 S1 Pages S7–S35) | Newcastle, UK |
| 2011 | S Cirak, V Arechavala-Gomeza, et al | 9th European Paediatric Neurology Society Congress | | Duvrovnik, Croatia |
| | S Cirak, V Arechavala-Gomeza et al. | 4rd UK Neuromuscular Translational Research Conference | Neuromuscular Disorders (Vol. 21, Pages S7-S8) | London, UK |
| 2010 | S. Cirak; L. Feng; S. Torelli; V. Arechavala-Gomeza; et al | 15th International Congress of the World Muscle Society | Neuromuscular Disorders Vol. 20, Page 585 | Kunamoto, Japan |
| | Jihee Kim, Virginia Arechavala-Gomeza, et al. | 3rd UK Neuromuscular Translational Research Conference | | Oxford, UK |
| | A.Malerba, P. Sharp, IR Graham, V Arechavala-Gomeza et al | 7th Annual Conference of the British Society for Gene Therapy | Human Gene Therapy 21(4): 507-525 | London, UK |
| 2009 | V. Arechavala-Gomeza, on behalf of the MDEX consortium | TREAT-NMD International Conference | | Brussels, Belgium |
| | V Arechavala-Gomeza, et al. | 2nd UK Neuromuscular Translational Research Conference | | Newcastle, UK |
| | V. Arechavala-Gomeza, et al | 14th International Congress of the World Muscle Society | Neuromuscular Disorders (Vol. 19, Issue 8, Page 615) | Geneva, Switzerland |
| 2008 | M. Kinali, V. Arechavala-Gomeza, et al. | 13th International Congress of the World Muscle Society | Neuromuscular Disorders (Vol. 18, Issue 9, Page 773) | Newcastle, UK |

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| | | British Paediatric Neurology Association Conference | Developmental Medicine & Child Neurology *50*, 6-32 | Leeds, UK |
| 2007 | V. Arechavala-Gomeza, Maria Kinali et al | 12th International Congress of the World Muscle Society | Neuromuscular Disorders (Vol. 17, Issue 9, Page 842) | Sicily, Italy |
| 2006 | V. Arechavala-Gomeza, et al | 11th International Congress of the World Muscle Society | Neuromuscular Disorders (Vol. 17, Issue 9, Page 842) | Brugges, Belgium |
| 2004 | V.Arechavala-Gomeza, et al. | 2nd Meeting of the European ALS/MND Consortium | | Nice, France |
| 2003 | Arechavala-Gomeza V | 14th International Symposium on ALS/MND | ALS and other motor neuron disorders 2003 4 (Suppl 1), 79–96 | Milan, Italy |
| 2002 | | 13th International Symposium on ALS/MND | | Melbourne, Australia |
| 2001 | | 12th International Symposium on ALS/MND | | Oakland, USA |

## CURRENT AND PAST FUNDING

### On-going Grants

| *Project Title* | *Funding source* | *Amount (Euros)* | *Period* | *Role of the PI* |
|---|---|---|---|---|
| Evaluación y desarrollo de terapias personalizadas para distrofias musculares por deficit de Colágeno VI | Ministerio de Ciencia e Innovación | 157300 | Sept 2022-Aug 2026 | PI: **Arechavala-Gomeza** |
| DARTER Scientific Matchmaking meeting | Cooperation in Science and Technology, European Union | 2000 | July 2022 | PI: **Arechavala-Gomeza** |
| DARTER Virtual Networking Grant | Cooperation in Science and Technology, European Union | 4000 | May 2022-Oct 2022 | PI: **Arechavala-Gomeza** |
| Identificación de síndromes de predisposición genética al cáncer infantil e implicación del splicing aberrante en su desarrollo | Departamento de Salud, Gobierno Vasco | 82.655 € | Jan 2022-Dec 2025 | Collaborator PI Olatz Villate |
| Desarrollo de una terapia frente a la distrofia miotónica tipo 1 con Ahulken | Departamento de Salud, Gobierno Vasco | 62.037 € | Jan 2021-Dec 2021 | Collaborator PI Ander Matheu |
| Desarrollo preclínico de un compuesto modulador de la utrofina para el tratamiento de las distrofias de Duchenne y Becker | Departamento de Salud, Gobierno Vasco | 9.103,76 | Jan 2021-Dec 2021 | PI: **Arechavala-Gomeza** |
| DARTER Virtual Networking Grant | Cooperation in Science and Technology, European Union | 4000 | July 2021-Oct 2021 | PI: **Arechavala-Gomeza** |
| Optimización de un protocolo de PCR digital para la cuantificación de la expresión de utrofina. | Departamento de Salud, Gobierno Vasco | 18.876 € | Jan 2020-Dec 2020 | PI: **Arechavala-Gomeza** |

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| Myoblots para la evaluación rápida de nuevos tratamientos para la distrofia miotónica (MaTILDa) | Departamento de Salud, Gobierno Vasco | 84.580€ | Jan 2020-Sept 2023 | PI: **Arechavala-Gomeza** |
| Rio Hortega Fellowship to Patricia Soblechero-Martín | Rio Hortega Fellowship Program (Instituto de Salud Carlos III) | 78.841€ | Jan 2020-Dec 2021 | PI: Soblechero-Martín, Director: Arechavala-Gomeza |
| COST Action CA17103 "Delivery of Antisense RNA Therapeutics" www.antisenserna.eu | Cooperation in Science and Technology, European Union | GP1: 150.000€<br>GP2: 130.000€<br>GP3: 110.170€<br>GP4: 149.585€<br>GP5: 57.500€ | Oct 2018-A 2023 | Main proposer and Chair **Arechavala-Gomeza** |
| Distrofia miotónica de inicio infantil y del adulto: Evaluación de nuevos tratamientos y patogenicidad a través de análisis genético, epigenético y de imagen molecular | Proyectos de Investigación en Salud (Instituto de Salud Carlos III) | 65.945€ | 2019-2021 | Collaborator. PI Gisela Nogales |
| Myoblots para la evaluación rápida de nuevos tratamientos para la distrofia miotónica (MaTILDa) | Proyectos de Investigación en Salud (Instituto de Salud Carlos III) | 81.070€ | Jan 2019-Jun 2023 | PI: **Arechavala-Gomeza** |
| Nueva aproximación terapéutica para las miopatías de Ullrich y Bethlem basada en la edición de mutaciones dominantes del colágeno VI. | CIBERER Acciones Cooperativas y Complementarias Intramurales 2018 (ACCI) | 49.500€ | 2019 | Colaborator. PI Cecilia Jimenez Mallebrera |
| Ikerbasque Starting Grant | Ikerbasque Basque foundation for Science | 30.000 € | 2019 | PI: **Arechavala-Gomeza** |
| Validación de un método de selección de fármacos moduladores de la expresión de utrofina | Departamento de Salud, Gobierno Vasco | 24.744€ | 2018 | PI: **Arechavala-Gomeza** |
| Miguel Servet Fellowship II | Miguel Servet Fellowship Program (Instituto de Salud Carlos III). | 91.125€ | June 2018-May 2021 | PI: **Arechavala-Gomeza** |
| Oligonucleotidos antisentido como nueva estrategia terapeutica en la miocardiopatia hipertrofica | Sociedad Española de Cardiología | 20.000€ | Oct 2017-sept 2018 | Colaborator PI: Maria Sabater Molina |
| CRISPR/Cas gene editing of animal model cultures for a faster transfer of DMD treatments to the clinic. | Duchenne Parent Project Spain | 70.000€ | Jan 2017-Dec 2019 | PI: **Arechavala-Gomeza** |
| Edición genética avanzada para el tratamiento de la Distrofia Muscular de Duchenne¨ | Proyectos de Investigación en Salud (Instituto de Salud Carlos III) | 136.427,5€ | Jan 2016-June 2020 | PI: **Arechavala-Gomeza** |

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| (ENiGMA: EdicioN Genetica MusculAr) | | | | |

**Past funded grant applications/ participation in research projects**

| *Project Title* | *Funding source* | *Amount (Euros)* | *Period* | *Role of the PI* |
|---|---|---|---|---|
| Cuantificación de utrofina en distrofias musculares. | Departamento de Salud del Gobierno Vasco | 39.800€ | Jan 2017-Dec 2018 | PI: **Arechavala-Gomeza** |
| "ANTeS: Advanced Neuromuscular Treatment Screening." | Marie Curie Career Integration Grant, FP7-PEOPLE-2013-CIG. | 100.000€ | Sept 2013-2017 | PI: **Arechavala-Gomeza** |
| QUADRES: QUAntification of Dystrophin Restoration by Exon Skippping | "Todos Somos Raros" Tele-marathon (Fundación Isabel Gemio, ASEM, FEDER) | 98.989€ | 2015-2017 | PI: **Arechavala-Gomeza** |
| "Translational research in neuromuscular disorders: advanced dystrophin quantification for streamlined screening of RNA treatments." | Miguel Servet Fellowship Program (Instituto de Salud Carlos III) | 335.500€+ 60.950€ | June 2013-May 2016 (-2018) | PI: **Arechavala-Gomeza** |
| COST Short Term Scientific Mission for Dr Iker García Jimenez | COST European Cooperation in Science and Technology. | 1200 € | 13/04/15-17/04/15 | PI: **Arechavala-Gomeza**. |
| COST Short Term Scientific Mission for Ms Estibaliz Ruiz Del Yerro | COST European Cooperation in Science and Technology. COST-STSM-BM1207-15608 | 1900 € | 1/12/13-6/12/13 | PI: **Arechavala-Gomeza.** |
| Student bench fees for Mr A Musawi. | MSc in Gene and Cell Therapy, University College London | £2.000 | April- July 2012 | PI: **Arechavala-Gomeza.** |
| "Advanced antisense oligonucleotide technology for exon skipping in Duchenne muscular dystrophy" | Funded by the Health Innovation Challenge Fund (Wellcome Trust and the UK Department of Health) | £2,468,621 | Mar 2011- Mar 2014 | Postdoctoral Research Associate PI: Francesco Muntoni. |
| "Accelerate screening of PPMO drug candidates targeting exons 53, 51, 45 and 44" | AVI BioPharma | | Mar 2010- Mar 2011 | Supervised the work of the research assistant in this project PI: Francesco Muntoni, |

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| "Antisense oligonucleotide to restore the reading time frame in Duchenne patients with out of frame duplications" | Gavriel Meir Trust | £480.000 | Oct 2008-Oct 2011 | Supervised the work of the research assistant responsible for this work PI: Francesco Muntoni |
| "Dose-Ranging Study of AVI-4658 to Induce Dystrophin Expression in Selected Duchenne Muscular Dystrophy (DMD) Patients". | UK Medical Research Council (MRC) and Sarepta Therapeutics | £800.000 | Jan 2009-Dec 2010, | Postdoctoral Research Associate. PI: Francesco Muntoni. |
| "Restoring Dystrophin Expression in Duchenne Muscular Dystrophy: A Phase I/II Clinical Trial Using AVI-4658", I was employed by the MDEX consortium | UK Department of Health | £1,600,000 | June 2005-Dec 2008 | Postdoctoral Research Associate PI: Francesco Muntoni. |
| "Creation of a national DNA Bank and cell line repository for functional genomic research into the aetiopathogenesis of MND" | MNDA and Wellcome Trust | £532,335 | 2003-2005 | Research Assistant PI: Christopher Shaw |
| "Molecular studies on mutant SOD1: the role of catalysis and aggregation in the pathogenesis of ALS" | Motor Neurone Disease Association (MNDA) PhD Studentship, | (University fees plus stipend.) | Oct 2000-Sept 2003 | PhD Student Supervisor: Christopher Shaw |