

# Virginia Arechavala-Gomez

## PERSONAL INFORMATION

Date of birth: 21 October 1975  
Web site: [www.arechavala-lab.com](http://www.arechavala-lab.com)  
Twitter account: [@VArechavala](https://twitter.com/VArechavala),  
Researcher identifier: ORCID: <http://orcid.org/0000-0001-7703-3255>

## 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

June 2013- Current **Group Leader** **Biocruces Bizkaia Health Research Institute**, Barakaldo, Spain  
*Neuromuscular Disorders Group. Currently 4 researchers.*  
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** Institute of Psychiatry, **Kings College London**, UK  
*Research on Amyotrophic Lateral Sclerosis*

## FELLOWSHIPS AND AWARDS

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-Sklodowska-Curie Fellowship**, Career Integration Grant, European Union

## MAIN RESEARCH PROJECTS/ FUNDING SUMMARY

Dr Arechavala-Gomez has been a PI since June 2013. In this period, she has led several research projects for the value of over 900.000€. In her previous positions, she was a senior postdoctoral researcher at a laboratory that led several large multi-centre grants and she contributed to the drafting of many of them. See FUNDING section for a detailed list of grants.

## CITATION REPORT

Sum of publications: 23 (12 as main author), h-index 19, g-index=25 Sum of times cited: 2455.  
Average of citations per paper: 102, including highly cited first author publications in Lancet (730), Lancet Neurology (644) and Human Gene Therapy (157) (Google Scholar, as of June 2018). For a detailed list, see PUBLICATIONS.

## NETWORKS

2018-2022 [COST Action CA17103](http://www.cost.eu/Action/CA17103) Main proposer/Chair. 26 countries, 8 companies, 3 NGOs.  
2013-2017. COST Action BM1207 ([www.exonskipping.eu](http://www.exonskipping.eu)), Management Committee Member.  
2007-current. Member, TREAT-NMD Network ([www.treat-nmd.eu](http://www.treat-nmd.eu))  
2005-2013 Postdoctoral researcher, MDEX consortium: "to promote collaborative translation research for Duchenne muscular dystrophy".

## **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](http://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](http://goo.gl/vbHE8))

## **TECHNOLOGY TRANSFER**

Developed a screening method 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**

**2018-Current** Member, PhD program, “Molecular biology and Biomedicine” University of the Basque Country (UPV/EHU), Spain.

**2016-current** Invited lecturer MSc Course “Master in Neurosciences” University of Barcelona, 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**

- 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
- 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-Gomez 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.

June 2018- current	Gabriela Gonzalez, Post-graduate Researcher, Biocruces
Dec 2017-July 2018	Irene Larrañaga, Master’s student, University of the Basque Country
Nov 2017-Current	Edurne Albiasu, Research Technician, Biocruces
July 2017-current	Patricia Soblechero, PhD Student, Biocruces
Dec 2016-June 2017	Alba Paramio, Master’s student, University of the Basque Country
March 2015-Dec 2017	Iker García Jimenez. Postdoctoral researcher, Biocruces
Dec 2013- Dec 2017	Estibaliz Ruiz del Yerro, Research technician, Biocruces
Dec 2012 -Mar 2013	Courtney Young, MRes in Biomedicine, University College London
2011-2012	Narinder Janghra, Research technician, University College London
April-July 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 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**

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) European 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>).  
Peer Reviewer for Nature Communications, Lancet Neurology, Human Molecular Genetics, PLOS One, Neuromuscular Disorders, Neuropathology and Applied Neurobiology, Experimental and Molecular Pathology, Human Mutation and New England Journal of Medicine (NEJM).

## **SCIENTIFIC SOCIETIES**

2015-current Spanish Society for Gene and Cell Therapy (SETGyC)  
2014-current. Marie Curie Alumni Association (MCAA) ([www.mariecuriealumni.eu](http://www.mariecuriealumni.eu))  
2014-current World Muscle Society ([www.wms.com](http://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](http://www.sruk.org.uk))

## **PUBLIC ENGAGEMENT**

Dr Arechavala-Gomez 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 of 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 Talenta (<http://goo.gl/nxz9rO>), Gioconda Salud and Jakin Mina programs with seminars to undergraduate students about scientific careers.

## **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

## PUBLICATIONS

Authors, title and date	Journal
Aartsma-Rus, A and <b>Arechavala-Gomez V.</b> News and Views: “Why dystrophin quantification is key in the eteplirsen saga” <b>July 2018</b>	Nature Reviews Neurology
Ruiz-Del-Yerro, E, Mamchoui, K, Garcia-Jimenez I, <b>Arechavala-Gomez V</b> <b>August 2018</b>	Neuropath Appl Neurobiol
Godfrey, C Goyenvalle A, ... <b>Arechavala-Gomez V</b> “ <i>Delivery is key: lessons learnt from developing splice switching antisense therapies</i> ” <b>May 2017</b>	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, <b>Arechavala-Gomez et al</b> “ <i>Policy view: Cooperation among stakeholders to overcome challenges in orphan medicine development The example of Duchenne muscular dystrophy</i> ” <b>July 2016</b>	Lancet Neurology
<b>Arechavala-Gomez V*</b> , Khoo B*, Aartsma-Rus A* “Exon skipping therapy in the treatment of genetic diseases” <b>*Equal contribution. December 2014</b>	The Application of Clinical Genetics
Anthony K*, <b>Arechavala-Gomez V*</b> , Taylor LE, et al. “ <i>Dystrophin quantification: biological and translational research implications</i> ” <b>*Equal contribution. Nov 2014</b>	Neurology
Anthony K, <b>Arechavala-Gomez V</b> , Ricotti V et al “ <i>Biochemical characterisation of patients with in- or out-of-frame DMD deletions pertinent to exon 44 or 45 skipping</i> ” <b>Jan 2014</b>	JAMA Neurology (Archives of Neurology)
Anthony K, Feng L, <b>Arechavala-Gomez V</b> , et al “ <i>Exon Skipping Quantification by qRT-PCR in Duchenne Muscular Dystrophy Patients Treated with the Antisense Oligomer Eteplirsen</i> ” <b>Nov 2012</b>	Human Gene Therapy Methods
<b>Arechavala-Gomez V</b> , Feng L, Morgan JE, et al. “ <i>Antisense Measuring Dystrophin-faster is not necessarily better.</i> ” CORRESPONDENCE. <b>August 2012</b>	Nature Reviews Neurology
<b>Arechavala-Gomez V</b> , Anthony K, Morgan J, et al “ <i>Antisense Oligonucleotide-Mediated Exon Skipping for Duchenne Muscular Dystrophy: Progress and Challenges.</i> ” <b>June 2012</b>	Current Gene Therapy
<b>Arechavala-Gomez V</b> , S Cirak, K Anthony, et al “ <i>Exon-skipping therapy for Duchenne muscular dystrophy - Authors' reply.</i> ” CORRESPONDENCE. <b>Jan. 2012</b>	Lancet.
Cirak S, Feng L, Anthony K, <b>Arechavala-Gomez V</b> , et al “ <i>Restoration of the Dystrophin-associated Glycoprotein Complex After Exon Skipping Therapy in Duchenne Muscular Dystrophy.</i> ” <b>Jan 2012</b>	Mol Ther
Anthony K, Cirak S, Torelli S, Tasca G, Feng L, <b>Arechavala-Gomez V</b> , et al “ <i>Dystrophin quantification and clinical correlations in Becker muscular dystrophy: implications for clinical trials.</i> ” <b>Nov 2011</b>	Brain
Cirak S*, <b>Arechavala-Gomez* V</b> , Guglieri M, et al “ <i>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.</i> ” <b>*Equal contribution. August 2011</b>	Lancet
Malerba A, Sharp PS, Graham IR, <b>Arechavala-Gomez V</b> , et al “ <i>Chronic systemic therapy with low-dose morpholino oligomers ameliorates the pathology and normalizes locomotor behavior in mdx mice.</i> ” <b>Feb 2011</b>	Molecular Therapy
Kinali M, <b>Arechavala-Gomez V</b> , Cirak S, et al “ <i>Muscle histology vs MRI in Duchenne muscular dystrophy.</i> ” <b>Jan 2011</b>	Neurology
<b>Arechavala-Gomez V</b> , Kinali M, Feng L, et al “ <i>Immunohistological intensity measurements as a tool to assess sarcolemma-associated protein expression.</i> ” <b>June 2010</b>	Neuropath Applied Neurobiology

Arechavala-Gomez V, Kinali M, Feng L, et al “ <i>Revertant fibres and dystrophin traces in Duchenne muscular dystrophy: implication for clinical trials.</i> ” May 2010	Neuromuscular Disorders
Popplewell LJ, Adkin C, Arechavala-Gomez V, Aartsma-Rus A, et al “ <i>Comparative analysis of antisense oligonucleotide sequences targeting exon 53 of the human DMD gene: Implications for future clinical trials.</i> ” Feb 2010	Neuromuscular Disorders
Meng J, Adkin CF, Arechavala-Gomez V, et al. “ <i>The contribution of human synovial stem cells to skeletal muscle regeneration</i> ” Jan 2010	Neuromuscular Disorders.
Walmsley GL, Arechavala-Gomez V, Fernandez-Fuente M, et al “ <i>A Duchenne muscular dystrophy gene hot spot mutation in dystrophin-deficient cavalier king charles spaniels is amenable to exon 51 skipping.</i> ” Jan 2010	PLoS One
Kinali M*, Arechavala-Gomez* V, Feng L, et al. “ <i>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.</i> ” *Equal contribution. Oct 2009	Lancet Neurology
Arechavala-Gomez V, Graham IR, Popplewell LJ, et al “ <i>Comparative analysis of antisense oligonucleotide sequences for targeted skipping of exon 51 during dystrophin pre-mRNA splicing in human muscle.</i> ” Sept 2007	Human Gene Therapy
CE Shaw, V Arechavala-Gomez, A Al-Chalabi. “ <i>Familial amyotrophic lateral sclerosis</i> ” Dec 2006. BOOK CHAPTER	Handbook of Clinical Neurology

## SELECTED MEETINGS

### Selected oral presentations

- 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 Letters, 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 talento en Bizkaia” Estrategia empresarial y diputación de Bizkaia, BIC Barakaldo April 2017
- Neurokafe, “Orphan drugs, rare diseases: a coffee break story” BioCruces Health 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
- 14<sup>th</sup> 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
2018	E Ruiz-Del-Yerro, P Soblechero-Martin, I Larrañaga, E Albiasu-Arteta, V Arechavala-Gomez	Neurogune		Vitoria, Spain
2018	P Soblechero-Martin, I García-Jiménez, E Ruiz-	Neurogune		Vitoria, Spain

	Del-Yerro, E Albiasu-Arteta, V Arechavala-Gomez			
2018	E Ruiz-Del-Yerro, P Soblechero-Martin, I Larrañaga, E Albiasu-Arteta, <u>V Arechavala-Gomez</u>	International Congress on Neuromuscular Disorders.	Journal of Neuromuscular Disorders, vol. 5, no. s1, pp. S1-S408, 2018 <a href="https://doi.org/10.3233/JND-189001">https://doi.org/10.3233/JND-189001</a>	Vienna, Austria.
2018	<u>P Soblechero-Martin</u> , I García-Jiménez, E Ruiz-Del-Yerro, E Albiasu-Arteta, V Arechavala-Gomez	International Congress on Neuromuscular Disorders.	Journal of Neuromuscular Disorders, vol. 5, no. s1, pp. S1-S408, 2018 <a href="https://doi.org/10.3233/JND-189001">https://doi.org/10.3233/JND-189001</a>	Vienna, Austria.
2017	<u>P. Soblechero-Martín</u> , E. Ruiz-Del-Yerro, I. García-Jimenez, V. Arechavala-Gomez.	Science + ERA Career Day,		Bilbao, Spain
2016	<u>I García</u> , E Ruiz-Del-Yerro, V Arechavala-Gomez	International Congress of the World Muscle Society	Neuromuscular Disorders, Vol. 26, S159-S160	Granada, Spain
2016	E Ruiz-Del-Yerro, <u>I García</u> , <u>V Arechavala-Gomez</u>	International Congress of the World Muscle Society	Neuromuscular Disorders, Vol. 26, S159	Granada, Spain
2016	E Ruiz-Del-Yerro, <u>I García</u> , <u>V Arechavala-Gomez</u>	Neurogune,		Bilbao, Spain
2015	M. Hiller, ... Aartsma-Rus, A. Ferlini, V. Arechavala-Gomez, N. Datson, P. Spitali	11th Annual Meeting Oligonucleotide Therapeutics Society,		Leiden, Netherlands
2015	E Ruiz-Del-Yerro, <u>I García</u> , <u>V Arechavala-Gomez</u>	International Congress of the World Muscle Society	Neuromuscular disorders October 2015, Volume 25, Supplement 2, , Page S254	Brighton, UK
2015	E Ruiz-Del-Yerro, <u>I García</u> , <u>V Arechavala-Gomez</u>	Science + Meeting		Bilbao, Spain
2014	<u>S. Torelli</u> , K Anthony, V Arechavala-Gomez, et al	UK Neuromuscular Translational Research Conference 2014	Neuromuscular disorders (March 2014 Volume 24, Supplement 1, Page S11)	London, UK
2013	<u>V Arechavala-Gomez</u> , <u>C Godfrey</u> , A Hibbert et al	UK Neuromuscular Translational Research Conference 2013		Oxford, UK
2012	<u>K Anthony</u> , V Arechavala-Gomez et al.	17 <sup>th</sup> International Congress of the World Muscle Society	Neuromuscular Disorders (Vol. 22, Pages 804-908)	Perth, Australia
	<u>L Popplewell</u> , V Arechavala-Gomez, et al <u>K Anthony</u> , S Cirak, S	UK Neuromuscular Translational Research Conference 2012	Two abstracts: Neuromuscular Disorders (Vol 22 S1	Newcastle, UK

	Torelli, G Tasca, L Feng, V Arechavala-Gomez, et al		Pages S7-S35)	
2011	S. Cirak, V Arechavala-Gomez, et al	9 <sup>th</sup> European Paediatric Neurology Society Congress		Duvrovnik, Croatia
	S. Cirak, V Arechavala-Gomez et al.	4 <sup>rd</sup> UK Neuromuscular Translational Research Conference	Neuromuscular Disorders (Vol. 21, Pages S7-S8)	London, UK
2010	S. Cirak; L. Feng; S. Torelli; V. Arechavala-Gomez; et al	15 <sup>th</sup> International Congress of the World Muscle Society	Neuromuscular Disorders Vol. 20, Page 585	Kunamoto, Japan
	Jihee Kim, Virginia Arechavala-Gomez, et al.	3 <sup>rd</sup> UK Neuromuscular Translational Research Conference		Oxford, UK
	A.Malerba, P. Sharp, IR Graham, V Arechavala-Gomez et al	7 <sup>th</sup> Annual Conference of the British Society for Gene Therapy	Human Gene Therapy 21(4): 507-525	London, UK
2009	V. Arechavala-Gomez, on behalf of the MDEX consortium	TREAT-NMD International Conference		Brussels, Belgium
	V. Arechavala-Gomez, et al.	2 <sup>nd</sup> UK Neuromuscular Translational Research Conference		Newcastle, UK
	V. Arechavala-Gomez, et al	14 <sup>th</sup> International Congress of the World Muscle Society	Neuromuscular Disorders (Vol. 19, Issue 8, Page 615)	Geneva, Switzerland
2008	M. Kinali, V. Arechavala-Gomez, et al.	13 <sup>th</sup> International Congress of the World Muscle Society	Neuromuscular Disorders (Vol. 18, Issue 9, Page 773)	Newcastle, UK
		British Paediatric Neurology Association Conference	Developmental Medicine & Child Neurology 50, 6-32	Leeds, UK
2007	V. Arechavala-Gomez, Maria Kinali et al	12 <sup>th</sup> International Congress of the World Muscle Society	Neuromuscular Disorders (Vol. 17, Issue 9, Page 842)	Sicily, Italy
2006	V. Arechavala-Gomez, et al	11 <sup>th</sup> International Congress of the World Muscle Society	Neuromuscular Disorders (Vol. 17, Issue 9, Page 842)	Brugges, Belgium
2004	V. Arechavala-Gomez, et al.	2 <sup>nd</sup> Meeting of the European ALS/MND Consortium		Nice, France
2003	Arechavala-Gomez V	14 <sup>th</sup> International Symposium on ALS/MND	ALS and other motor neuron disorders 2003 4 (Suppl 1), 79-96	Milan, Italy
2002		13 <sup>th</sup> International Symposium on ALS/MND		Melbourne, Australia
2001		12 <sup>th</sup> International Symposium on ALS/MND		Oakland, USA



## CURRENT AND PAST FUNDING

### On-going Grants

<i>Project Title</i>	<i>Funding source</i>	<i>Amount (Euros)</i>	<i>Period</i>	<i>Role of the PI</i>
COST Action CA17103 "Delivery of Antisense RNA Therapeutics"	Cooperation in Science and Technology, European Union		Oct 2018-Oct 2022	Main proposer <b>Arechavala-Gomez</b>
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-Dec 2021	PI: <b>Arechavala-Gomez</b>
Miguel Servet Fellowship II	Miguel Servet Fellowship Program (Instituto de Salud Carlos III).	91.125€	June 2018-May 2021	PI: <b>Arechavala-Gomez</b>
Oligonucleotidos antisentido como nueva estrategia terapeutica en la miocardiopatía hipertrofica	Sociedad Española de Cardiología	20.000€	Oct 2017-sept 2018	Colaborator PI: Maria Sabater Molina
Cuantificación de utrofina en distrofias musculares.	Departamento de Salud del Gobierno Vasco	39.800€	Jan 2017-Dec 2018	PI: <b>Arechavala-Gomez</b>
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 2018	PI: <b>Arechavala-Gomez</b>
Edición genética avanzada para el tratamiento de la Distrofia Muscular de Duchenne (ENiGMA: Edición Genética Muscular)	Proyectos de Investigación en Salud (Instituto de Salud Carlos III)	136.427,5€	Jan 2016-Dec 2018	PI: <b>Arechavala-Gomez</b>

Past funded grant applications/ participation in research projects

<i>Project Title</i>	<i>Funding source</i>	<i>Amount (Euros)</i>	<i>Period</i>	<i>Role of the PI</i>
“ANTeS: Advanced Neuromuscular Treatment Screening.”	Marie Curie Career Integration Grant, FP7-PEOPLE-2013-CIG.	100.000€	Sept 2013-2017	PI: <b>Arechavala-Gomez</b>
QUADRES: QUAntification of Dystrophin Restoration by Exon Skipping	“Todos Somos Raros” Tele-marathon (Fundación Isabel Gemio, ASEM, FEDER)	98.989€	2015-2017	PI: <b>Arechavala-Gomez</b>
“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: <b>Arechavala-Gomez</b>
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: <b>Arechavala-Gomez</b> .
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: <b>Arechavala-Gomez</b> .
Student bench fees for Mr A Musawi.	MSc in Gene and Cell Therapy, University College London	£2.000	April- July 2012	PI: <b>Arechavala-Gomez</b> .
“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,

“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 ( <a href="http://goo.gl/6k0IQ">http://goo.gl/6k0IQ</a> )	£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 ( <a href="http://goo.gl/fXuB4">http://goo.gl/fXuB4</a> )	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