

Curriculum Vitae

Name: CRISTINA LIBERATI, MD
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Work Email: cristina.liberati@childrens.harvard.edu
Place of Birth: Rome, Italy

Appointments at Hospital/Affiliated Institutions:

07/2021 - ongoing	Resident PGY1 BCRP	Department of Pediatrics Child Neurology Pathway	Boston Children's Hospital Boston Medical Center
09/2016 - 07/2021	Researcher /Post- Doctorate Associate	Department of Pediatrics, Health Science Center	University of Florida, Gainesville, FL, USA

Education:

10/2008 - 11/2015	MD, graduated with honors	Medicine and Surgery	Sapienza University of Rome, Rome, Italy
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Final Thesis:

Experimental thesis project named "E.N.A. 14 (Epilepsy in patients above 14 years old) Test: Finding The Source of the Stigma in Teenagers with Epilepsy". E.N.A. 14 is a new test created ad hoc to be administered to teenagers with epilepsy to quantify the level and the source of stigma related to their diagnosis. Currently the test is being used by 7 different health centers spread around Italy to be standardized soon.

Postdoctoral Training:

01/2016 - 02/2016	Observership	Department of Pediatrics, Division of Genetics and Metabolism	University of Florida, Gainesville, FL, USA
04/2016	Post-graduation Internship (Italian Medical License State Exam)	Emergency Medicine / ER - Emergency Room / Short Stay Observation and Short Recovery Units/First Aid / Internal Medicine	Azienda Ospedaliero- Universitaria Sant'Andrea - Rome, Italy
05/2016	Post-graduation Internship (Italian Medical License State Exam)	Emergency Surgery / General Surgery	Azienda Ospedaliero- Universitaria Sant'Andrea - Rome, Italy

06/2016	Post-graduation Internship (Italian Medical License State Exam)	PCP office/Family Practice	PCP Office, Anguillara Sabazia (RM), Italy
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Research Clinical Trials Involvement (09/2016 – 05/2021):

<ul style="list-style-type: none"> ASPIRO: A Phase 1 / 2 Randomized, Open-Label, Ascending-Dose, Delayed- Treatment Concurrent Control Clinical Study to Evaluate the Safety and Preliminary Efficacy of AT132, an AAV8-Delivered Gene Therapy in X-Linked Myotubular Myopathy (XLMTM) Patients.
<ul style="list-style-type: none"> INCEPTUS: A Prospective, Non-Interventional Clinical Assessment Study in X-Linked Myotubular Myopathy (XLMTM) Subjects Aged 3 Years and Younger
<ul style="list-style-type: none"> CAP-1002-DMD-01 (HOPE): A Randomized, Open-label Study of the Safety and Efficacy of Multi-Vessel Intracoronary Delivery of Allogeneic Cardiosphere-Derived Cells in Patients with Cardiomyopathy Secondary to Duchenne Muscular Dystrophy [HOPE-Duchenne (Halt cardiomyopathy progression in Duchenne)]
<ul style="list-style-type: none"> Open-Label Extension of the Halt Cardiomyopathy Progression in Duchenne (Hope-Duchenne) Trial
<ul style="list-style-type: none"> Open-Label, Multiple-Dose, Efficacy, Safety, and Tolerability Study of Eteplirsen in Subjects with Duchenne Muscular Dystrophy Who Participated in Study 4658-us-201
<ul style="list-style-type: none"> An Open-Label, Multi-Center Study to Evaluate the Safety, Efficacy and Tolerability of Eteplirsen in Early-Stage Duchenne Muscular Dystrophy
<ul style="list-style-type: none"> An Open-Label, Multicenter, Study with a Concurrent Untreated Control Arm to Evaluate the Efficacy and Safety of Eteplirsen in Duchenne Muscular Dystrophy
<ul style="list-style-type: none"> A Phase 1 Trial to Evaluate the Safety, Tolerability, and Pharmacokinetics of a Single Dose of SRP-5051 in Patients with Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping Treatment
<ul style="list-style-type: none"> An Open-Label Extension Study for Patients with Duchenne Muscular Dystrophy Who Participated in Studies of SRP-5051
<ul style="list-style-type: none"> A Phase IIa Open-Label, Multiple Ascending Dose Study to Assess the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics, and Exploratory Efficacy of Vamorolone in Boys with Duchenne Muscular Dystrophy (DMD)
<ul style="list-style-type: none"> A Phase II, Dose Finding Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of NS-065/NCNP-01 in Boys with Duchenne Muscular Dystrophy (DMD)
<ul style="list-style-type: none"> A Prospective Non-Interventional Study in Subjects with Late-onset Pompe Disease who are Currently Being Treated with Enzyme Replacement Therapy
<ul style="list-style-type: none"> Longitudinal evaluation of anti-AAV antibodies in individuals with rare genetic diseases
<ul style="list-style-type: none"> Evaluation of Re-administration of Recombinant Adeno-Associated Virus Acid Alpha- Glucosidase (rAAV9-DES-hGAA) in Patients with Late-Onset Pompe Disease (LOPD)
<ul style="list-style-type: none"> An Open-Label, Fixed-Sequence, Ascending-Dose, First-in-Human Study to Assess the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics, and Efficacy of Intravenous Infusions of ATB200 Co-administered with Oral AT2221 in Adult Subjects with Pompe Disease
<ul style="list-style-type: none"> A Phase 3 Double-blind Randomized Study to Assess the Efficacy and Safety of Intravenous ATB200 Co-Administered with Oral AT2221 in Adult Subjects with Late-Onset Pompe Disease Compared with Alglucosidase Alfa/Placebo Capsule
<ul style="list-style-type: none"> An Open-label Study of the Pharmacokinetics, Safety, Efficacy, and Pharmacodynamics of ATB200/AT2221 in Pediatric Subjects aged 12 to < 18 years with Late-onset Pompe Disease
<ul style="list-style-type: none"> A Phase 3 Open-label Extension Study to Assess the Long-term Safety and Efficacy of Intravenous ATB200 Co-Administered with Oral AT2221 in Adult Subjects with Late-onset Pompe Disease
<ul style="list-style-type: none"> Barth Syndrome Registry and DNA Bank

<ul style="list-style-type: none"> • A Multi-Site, Randomized, Placebo-Controlled, Double-Blind, Multiple Ascending Subcutaneous Dose Study to Evaluate the Safety, Tolerability and Pharmacokinetics of RO7239361 in Ambulatory Boys with Duchenne Muscular Dystrophy
<ul style="list-style-type: none"> • Randomized, Double Blind, Placebo-Controlled, Study to Assess the Efficacy, Safety, and Tolerability of RO7239361 in Ambulatory Boys with Duchenne Muscular Dystrophy
<ul style="list-style-type: none"> • Expanded Access Trial of Systemic Delivery of Aspartoacylase ASPA (rAAV9-CB6-AspA) Gene Vector in a Single Patient with Canavan Disease
<ul style="list-style-type: none"> • Diet and Exercise in Pompe Disease
<ul style="list-style-type: none"> • A Double-Blind, Placebo-Controlled, Multicenter Study with an Open-Label Extension to Evaluate the Efficacy and Safety of SRP-4045 and SRP-4053 in Patients with Duchenne Muscular Dystrophy
<ul style="list-style-type: none"> • Long-term, Open-label Extension Study for Patients with Duchenne Muscular Dystrophy Enrolled in Clinical Trials Evaluating Casimersen or Golodirsen
<ul style="list-style-type: none"> • Biomarkers in Friedreich's Ataxia
<ul style="list-style-type: none"> • A Long-term Study to Evaluate Growth and Development Outcomes in Patients with Infantile-Onset Pompe Disease Who Are Receiving Alglucosidase Alfa
<ul style="list-style-type: none"> • Randomized, double blind, placebo controlled, multicenter study to evaluate the efficacy and safety of givinostat in ambulant patients with Duchenne Muscular Dystrophy
<ul style="list-style-type: none"> • Muscular Dystrophy Association Neuromuscular Observational Research (MOVR) Data Hub Protocol
<ul style="list-style-type: none"> • Rare Disease Longitudinal Observational Study
<ul style="list-style-type: none"> • Outcome Data and Specimen Bank for Patients with Rare Diseases and Myopathies: The Rare Disease Registry
<ul style="list-style-type: none"> • A randomized, controlled, open-label, single-ascending dose, phase I/II study to investigate the safety and tolerability, and efficacy of intravenous SGT-001 in male adolescents and children with Duchenne muscular dystrophy
<ul style="list-style-type: none"> • A Pilot Study of the Effects of Oral Administration of Zavesca® on Anti-rhGAA Immune Response in Subjects with Pompe Disease Receiving rhGAA Enzyme Replacement Therapy
<ul style="list-style-type: none"> • A Phase 2b/3 Prospective, Randomized, Double-Blind, Sham-Controlled Trial of VTS-270 (2-hydroxypropyl-β-cyclodextrin) in Subjects with Neurologic Manifestations of Niemann-Pick Type C1 (NPC1) Disease
<ul style="list-style-type: none"> • Open label, long-term safety, tolerability, and efficacy study of GIVINOSTAT in all DMD patients who have been previously treated in one of the GIVINOSTAT studies.
<ul style="list-style-type: none"> • Dynamic Respiratory Muscle Function in Late-Onset Pompe Disease
<ul style="list-style-type: none"> • Respiratory Muscle Function in Untreated X-linked Myotubular Myopathy
<ul style="list-style-type: none"> • Assessment of Cardiopulmonary Function in Duchenne Muscular Dystrophy
<ul style="list-style-type: none"> • Response to Diaphragmatic Pacing in Subjects with Pompe disease
<ul style="list-style-type: none"> • Respiratory Muscle Strength and Function in Patients with Neuromuscular Disease
<ul style="list-style-type: none"> • A Long-term Observational Study Evaluating Sarepta Therapeutics, Inc.'s Exon-Skipping Therapies in Patients with Duchenne Muscular Dystrophy under Conditions of Routine Clinical Practice
<ul style="list-style-type: none"> • Phase I-II Trial of Systemic Delivery of Recombinant Adeno-Associated Virus Acid Alpha-Glucosidase (rAAV9-DES-GAA) in Children with Early Onset Pompe Disease (<i>pending approval</i>)
<ul style="list-style-type: none"> • An expanded access study to investigate the safety, tolerability, and efficacy of PR001A in infants with Type II Gaucher Disease (<i>pending approval</i>)

Professional Societies:

2020 - Current	WMA	World Medical Association
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Honors, Prizes and Scholarships:

2008 - 2009	“Laziodisu”	Regional Scholarship	Regione Lazio, Rome, Italy
2009 - 2010	“Laziodisu”	Regional Scholarship	Regione Lazio, Rome, Italy
2010 - 2011	“Laziodisu”	Regional Scholarship	Regione Lazio, Rome, Italy
2011 - 2012	“Laziodisu”	Regional Scholarship	Regione Lazio, Rome, Italy
2012 - 2013	“Laziodisu”	Regional Scholarship	Regione Lazio, Rome, Italy
2013 - 2014	“Laziodisu”	Regional Scholarship	Regione Lazio, Rome, Italy
2014 - 2015	“Laziodisu”	Regional Scholarship	Regione Lazio, Rome, Italy
09/2012 - 02/2013	Erasmus Program	Exchange Program Scholarship	Sapienza, University of Rome, Italy
24/11/2015	Hippocratic Oath Reader	Graduation Session	Sapienza, University of Rome, Italy

Current Licensure and Certification:

07/2016 - Current	Italian Medical License # 62570	Ordine dei Medici, Albo dei Medici-Chirurghi di Roma (Italian Board of Physicians)	Italian Board of Physicians, Rome, Italy
10/2020	PALS	Pediatric Advanced Life Support Training - American Heart Association (AHA) Program	UF Health Shands CPR (Cardiopulmonary Resuscitation) Training Center, Gainesville, FL, USA
10/2020	ASLS	Advanced Cardiovascular Live Support Training - American Heart Association (AHA) Program	UF Health Shands CPR (Cardiopulmonary Resuscitation) Training Center, Gainesville, FL, USA
10/2020	BLS	Basic Life Support Training - American Heart Association (AHA) Program	UF Health Shands CPR (Cardiopulmonary Resuscitation) Training Center, Gainesville, FL, USA

Educational Material for Patients and Community:

07/19/2018	Gene Therapy: Your Questions Answered (Video)	Liberati, C., & Crowley, E., in collaboration with National Organization for Rare Disorders (NORD) - RareEDU educational program.	https://rarediseases.org ; https://youtu.be/5ChXI6cSQs0
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Publications and Newsletter:

06/2016	A Phenotypic Review of Connective Tissue and Related Disorders - Vol. XXVII No. 2; June 2016	Liberati C	The Raymond C. Philips Research and Education Unit (R.C.P.U.) - Division of Genetics and Metabolism - Department of Pediatrics - University of Florida
11/2017	Safety of Intradiaphragmatic Delivery of Adeno-Associated Virus-Mediated Alpha-Glucosidase (rAAV1-CMV-hGAA) Gene Therapy in children affected by Pompe Disease	Corti M, Liberati C , Smith BK, Lawson LA, Tuna IS, Conlon TJ, Erger KE, Islam S, Herzog R, Fuller DD, Collins S, Byrne BJ	Hum Gene Ther Clin Dev. doi: 10.1089/humc.2017.146
03/2019	Muscle Gene Therapy - Part III – Clinical Muscle Gene Therapy, Chapter 44, pg. 759 -774 – <i>Clinical Gene Therapy Trials for Pompe Disease</i>	Cristina Liberati , Stephanie Salabarria, Manuela Corti Barry J. Byrne	By Dongsheng Duan, Jerry R. Mendell; Springer International Publishing
03/2019	Muscle Gene Therapy - Part II – Preclinical Muscle Gene Therapy, Chapter 33, pg. 579-596 – <i>Preclinical Gene Therapy Studies for Metabolic Myopathy</i>	Stephanie Salabarria, Barry J. Byrne, Cristina Liberati , Manuela Corti	By Dongsheng Duan, Jerry R. Mendell; Springer International Publishing

Oral Presentations, Poster Presentations and Exhibits Presented at Professional Meetings:

02/27/2017	Poster Presentation: “Clinical Outcome measures of Efficacy in Treatment of Friedreich’s Ataxia”	2017 College of Medicine Research Poster Session	University of Florida, Gainesville, FL, USA
03/17/2017	Poster Presentation: “Clinical Outcome Measures of Efficacy in Treatment of Friedreich’s Ataxia”	12th Annual Neuromuscular Plasticity Symposium	University of Florida, Gainesville, FL, USA
03/19/2017 to 03/22/2017	Poster Presentation: “Clinical Outcome Measures of Efficacy in Treatment of Friedreich’s Ataxia”	2017 MDA (Muscular Dystrophy Association) Scientific Conference	Arlington, VA, USA
05/18/2017	“Cross Reactive Immunologic Material (CRIM) Scale for Pompe Disease to Predict Disease Severity and the Need for Immunomodulation During Enzyme Replacement Therapy”	35th Annual Pediatric Science Day	University of Florida, Gainesville, FL, USA

06/05/2017	Poster Presentation: “Clinical Outcome Measures of Efficacy in Treatment of Friedreich’s Ataxia”	CTSI (Clinical and Translational Science Institute) Research Day	University of Florida, Gainesville, FL, USA
02/05/2018	“Coordination of Breath and Stimulator Initiation in Diaphragm-Paced Individuals with Pompe Disease”	14th Annual WORLD Symposium	San Diego, CA, USA
02/05/2018	“Immune Blockade in CNS Gene Therapy Improves Safety and Clinical Outcome”	14th Annual WORLD Symposium	San Diego, CA, USA
03/11/2018 to 03/14/2018	“Clinical Outcome Measures of Efficacy in Treatment of Friedreich’s Ataxia”	2018 MDA Scientific Conference	Arlington, VA, USA
03/11/2018 to 03/14/2018	Poster Presentation: “Diaphragm Pacing and Spontaneous Breath Coordination in Pompe Disease: a Case Report”	2018 MDA Scientific Conference	Arlington, VA, USA
03/16/2018	Poster Presentation: “Diaphragm Pacing and Spontaneous Breath Coordination in Pompe Disease: A Case Report”	13th Annual Neuromuscular Plasticity Symposium	University of Florida, Gainesville, FL, USA
05/16/2018 To 05/19/2018	“Immune Blockade in CNS Gene Therapy Improves Safety and Clinical Outcome”	ASGCT (American Society of Gene and Cell Therapy) 21st Annual Meeting	Chicago, IL, USA
09/28/2018	Poster Presentation: “Clinical Outcome Measures of Efficacy in Treatment of Friedreich’s Ataxia”	The FA Biomarkers and Outcome Measures Meeting	Tampa, FL, USA
02/07/2018	Poster Presentation: “Gene therapy as potential treatment for Canavan disease”	1st Annual Institute for Child Health Policy (IHP) - Pediatrics Research	University of Florida, Gainesville, FL, USA
02/19/2018	Poster Presentation: “Gene therapy as potential treatment for Canavan disease”	2019 College of Medicine Research Poster Session	University of Florida, Gainesville, FL, USA
04/13/2019 to 04/17/2019	Poster Presentation: “Friedreich’s Ataxia and Exercise Capacity”	2019 MDA Clinical and Scientific Conference	Orlando, FL, USA

06/13/2019	Poster Presentation: Motor Performance and Fatigue Self-Report in Carriers of XLMTM	37th Annual Pediatric Science Day	University of Florida, Gainesville, FL, USA
07/19/2019 to 07/21/2019	Oral Presentation: “Motor Performance and Fatigue Self-Report in Carriers of XLMTM”	6th Myotubular Myopathy and Centronuclear Myopathy Family Conference	St. Louis, MO, USA
11/28/2019 to 11/30/2019	“Valutazione della percezione e della conoscenza dell’epilessia: studio condotto su studenti e docenti di una scuola superiore italiana” (Evaluation of perception and knowledge of epilepsy: high school students and professors research study)	XLIV SINP (Italian Pediatric Neurology Society) National Congress	Naples, Italy
02/25/2020	Poster Presentation: “Canavan disease and gene therapy”	2020 College of Medicine Research Poster Session	University of Florida, Gainesville, FL, USA
02/27/2020	“Characterization of the cardiac and metabolic impairments in Friedreich’s Ataxia”	2nd Annual Institute for Child Health Policy (IHP) - Pediatrics Research	University of Florida, Gainesville, FL, USA
02/27/2020	Poster Presentation: “Canavan disease and gene therapy”	2nd Annual IHP - Pediatrics Research	University of Florida, Gainesville, FL, USA
02/28/2020	“Characterization of the cardiac and metabolic impairments in Friedreich’s Ataxia”	Rare Disease Day	University of Florida, Gainesville, FL, USA
05/2020	“Canavan disease and gene therapy”	2020 MDA Clinical and Scientific Conference	<i>Virtual due to COVID19 Pandemic</i>

Additional Courses:

10/2012 to 12/2012	Spanish language course for foreigners	Estudios Hispánicos	University of Barcelona, Barcelona Spain
01/2018 to 03/2018	Scholarly Writing Course	English Language Institute	University of Florida, Gainesville, FL, USA
02/2018 to 04/2018	English Conversation Class	English Language Institute	University of Florida, Gainesville, FL, USA

09/2019 to 11/2019	Fall 2019 Daily Writing Boot-Camp for UF Postdocs	English Language Institute	University of Florida, Gainesville, FL, USA
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Additional Experiences and Volunteering:

2002	Scuba diving certificate	Professional Association of Diving Instructors (PADI)	Sharm El Sheikh, Egypt
2002 to 2003	Goldsmithing and lost-wax casting course	San Francesco Middle School	Anguillara Sabazia, RM, Italy
03/2006 to 09/2017	Volunteer	Italian Red Cross (IRC)	Anguillara Sabazia, RM, Italy
06/2006 to 08/2006	Volunteer	Play Park	Anguillara Sabazia, RM, Italy
06/2006 to 03/2007	Volunteer	CEOS (Cooperative Educators Social Workers)	Anguillara Sabazia, RM, Italy
06/2007 to 08/2007	Entertainer/Hostess/Sport and dance instructor	Soglia Hotel Group, Scanzano Jonico	Scanzano Jonico, MT, Italy
10/2012 to 09/2015	Member/Volunteer	SISM – Italian Secretary of Medical Students	Rome, Italy
10/2018 to 10/2018	Volunteer	Child Life – UF Health- Shands Children’s Hospital	Gainesville, FL, USA
05/2019 to 05/2019	Participant	MDA (Muscular Dystrophy Association) Muscle Walk	Gainesville, FL, USA
07/2019 to 08/2019	Volunteer	MDA (Muscular Dystrophy Association) Camp	Umatilla (Orlando), FL, USA
11/2019 to 11/2019	Participant	FARA (Friedreich's Ataxia Research Alliance)	Clermont (Orlando), FL, USA

Languages:

Mother Language:	Italian	Native
Other Languages:	English	Advanced
	Spanish	Professional

Computer Skills:

Microsoft Office™ tools (Word, Excel, etc.)	Excellent
Adobe	Excellent
EPIC	Excellent
Internet and email management	Excellent
LabChart	Excellent
Endnote	Excellent
Prism	Very Good
Photoshop	Very Good

Driver's License:

Italian driver license – Type B:	Since 2007
Florida driver license – Class E:	Since 2016