



Academic Personnel Short Profile / Short CV

Institution:	The Cyprus Institute of Neurology & Genetics
Surname:	Lederer
Name:	Carsten Werner
Rank/Position:	Associate Professor
Program of Study:	MSc in Molecular Medicine / MSc in Medical Genetics / MSc in Neuroscience / MSc in Biomedical Research / MSc in Biotechnology / PhD in Molecular Medicine / PhD in Medical Genetics / PhD in Neuroscience
Scientific Domain: *	Molecular Genetics

Academic qualifications (list by highest qualification)				
Qualification	Year	Awarding Institution	Department	Thesis title
PhD Biology (8/7/2003)	1998 – 2003	University of East Anglia, Norwich, UK	The John Innes Centre, Virology Department	Regulation of Plant Gene Expression in Compatible Plant/Virus Interaction
BSc/MSc (“Diplom”) Molecular Biology (23/09/1998)	1993 – 1998	Friedrich-Wilhelm University, Bonn, Germany	Faculty of Natural Sciences, Biology Department	Constructing a Minimal Potatovirus-X Amplicon
German A-level leaving certificate (“Abitur”)	1983 – 1992	Grammar School Werner-Heisenberg, Neuwied, Germany	Not applicable	Not applicable



Employment history in Academic Institutions/Research Centers – List by the three (3) most recent

Period of employment		Employer	Location	Position
02/05/2023	present	The Cyprus Institute of Neurology & Genetics	Molecular Genetics Thalassaemia Department; also acting as PI of research group: <i>Genome Editing and Therapy</i>	Senior Scientist / Head of Department
01/07/2020	01/05/2023	The Cyprus Institute of Neurology & Genetics (Head of Dept. Marina Kleanthous)	Molecular Genetics Thalassaemia Department (with Dr. M. Kleanthous, CING, Nicosia, Cyprus), PI of research group: <i>Genome Editing and Therapy</i>	Scientist
01/01/2020	30/06/2020	As above	As above	Associate Scientist
01/10/2009	31/12/2019	As above	As above	Laboratory Scientific Officer

Key refereed journal papers, monographs, books, conference publications etc. List the five (5) more recent and other five (5) selected –(max total 10)

Ref. Number	Year	Title	Other authors	Journal and Publisher/ Conference	Vol.	Pages
* Corresponding author; + Shared last authorship						
1	2023	Progress and Harmonization of Gene Editing to Treat Human Diseases (Proceeding of COST Action CA21113 GenE-HumDi)	Cavazza A, Hendel A, Bak RO, Rio P, Güell M, Lainšček D, Arechavala-Gomez V, Peng L, Zehra Hapil F, Harvey J, Ortega FG, Gonzalez-	Molecular Therapy – Nucleic Acids	(MTNA-D-23-00535R2)	Accepted



			Martinez C, Lederer CW , Mikkelsen K, Gasiunas G, Kalter N, Gonçalves M, Garanto A, Montoliu L, Miccio A, Martin F, Cathomen T, Luo Y, Tsai QS, Benabdellah K.			
2	2023	High-efficiency editing in hematopoietic stem cells and the HUDEP-2 cell line based on in vitro mRNA synthesis	Papaioannou NY, Patsali P, Naiisseh B, Papasavva PL, Koniali L, Kurita R, Nakamura Y, Christou S, Sitarou M, Mussolino C, Cathomen T, Kleanthous M, Lederer CW	Front Genome Ed	5	1141618
3	2022	The optimized γ-globin lentiviral vector GGHI-mB-3D leads to potentially therapeutic HbF levels in vitro in CD34+ cells from sickle cell disease patients	Drakopoulou E, Georgomanoli M, Lederer CW , Panetsos F, Kleanthous M, Voskaridou E, Valakos D, Papanikolaou E, Anagnou NP	Viruses	14(12)	2716
4	2022	Catching Them Early: Framework Parameters and Progress for Prenatal and Childhood Application of Advanced Therapies	Lederer CW [*] , Koniali L, Buerki-Thurnherr T, Papasavva PL, La Grutta S, Licari A, Staud F, Bonifazi D, Kleanthous M	Pharmaceutics	14(4)	793
5	2022	Global Globin Network Consensus Paper: Classification and Stratified Roadmaps for	Halim-Fikri BH ⁺ , Lederer CW ⁺ [...], Kountouris P, Kleanthous M, Ramesar R, Zilfalil BA	J Pers Med	12(4)	552



		Improved Thalassaemia Care and Prevention in 32 Countries				
6	2022	CRISPR editing enables consequential tag-activated mi-croRNA-mediated endogene deactivation	Papasavva PL, Patsali P, Loucari CC, Kurita R, Nakamura Y, Sitarou M, Christou S, Kleanthous M ⁺ , Lederer CW⁺⁺	Int J Mol Sci	23(3)	1082
7	2021	Regulation of ER Composition and Extent, and Putative Action in Protein Networks by ER/NE Protein TMEM147	Maimaris G, Christodoulou A, Santama N, Lederer CW[*]	Int J Mol Sci	22(19)	10231
8	2021	Adapting the ACMG/AMP variant classification framework: a perspective from the ClinGen Hemoglobinopathy Variant Curation Expert Panel	Kountouris P, Stephanou C, Lederer CW , Synodinos JT, Bento C, Hartevelde CL, Fylaktou E, Koopmann TT, Fikri HH, Michailidou K, Nfonsam LE, Waye JS, Zilfalil BA, Kleanthous M; ClinGen Hemoglobinopathy Variant Curation Expert Panel	Hum Mutat	doi: 10.1002/ humu.242 80.	
9	2021	Therapy Development by Genome Editing of Hematopoietic Stem Cells	Koniali L, Lederer CW⁺⁺ , Kleanthous M ⁺	MDPI Cells	10(6)	1492
10	2021	Distinct miRNA Signatures and Networks Discern Fetal from Adult Erythroid Differentiation	Papasavva PL, Papaioannou NY, Patsali P, Kurita R, Nakamura Y, Sitarou M,	Int J Mol Sci	22(7)	3626



		and Primary from Immortalized Erythroid Cells	Christou S, Kleanthous M ⁺ , Lederer CW ⁺⁺			
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Exhibitions (where applicable). List the five (5) more recent and other five (5) selected. (max total 10)					
Ref. Number	Date	Topic	International / Local	Location*	Role in Exhibition
1	26/05/2023	6 th International conference organised by the Hellenic Society of Gene Therapy and Regenerative Medicine	International	Athens, Greece	Member of the Board and Organizing Committee. In-person. Invited speaker: Gene editing: CRISPR and beyond [in Greek]. Additional short oral abstract presentation. Co-author on two posters.
2	19/05/2023	Norway Grants Bilateral Action	International	Oslo, Norway	In-person. Invited speaker: Use cases for therapy development and reverse genetics by genome editing in hematopoietic cells (a Cypriot/CING perspective).
3	29/03/2023	Local Development and Poverty Reduction Event, Directorate General for Growth Ministry of Finance.	Local	Nicosia, Cyprus	In-person. Invited speaker: New infrastructure for



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					diagnosis and treatment of patients.
4	13/03/2023	1 st Meeting & Workshop COST Action 21113 GenE-HumDi	International	Granada, Spain	In-person. Invited speaker: Use cases for therapy development and reverse genetics by genome editing in hematopoietic cells (a Cypriot/CING perspective).
5	04/11/2022	10 th International Multithematic Bio-Medical Congress, organised by the European University of Cyprus, Nicosia, Cyprus.	International	Nicosia, Cyprus	In-person. Invited Lecture: Novel Tools and Approaches for Translational Research in Haematopoietic Cells and Beyond.
6	29/09/2022	ITHANET Information Day	International	Nicosia, Cyprus	Virtual. Invited Lecture: Global Globin Network Consensus Paper: Classification and Stratified Roadmaps for Improved Thalassaemia Care and Prevention in 32 Countries.
7	27/05/2022	5 th Congress of the Hellenic Society of Gene Therapy and Regenerative Medicine	International	Thessaloniki, Greece	In-person. Invited Lecture: Gene editing: off-target consequences.



8	09/04/2022	11 th Cyprus Thalassaemia Conference	Local	Nicosia, Cyprus	Virtual. Invited Lecture: Οι βασικές αρχές και η κλινική εφαρμογή της γονιδιακής θεραπείας για τη β-θαλασσαιμία.
9	08/12/2021	Sickle Cell Interest Group Meeting. Organised by the John Strouboulis Lab at King's College London	International	London, UK	Virtual. Invited Lecture: Gene Editing, Therapy and Regulation in Erythroid Cells.
10	26/11/2021	One-day Web Event – Gene and Cell Therapies: New Perspectives for old Diseases. Organised by the Hellenic Society of Gene Therapy and Regenerative Medicine,	International	Athens, Greece	Virtual. Invited Lecture: Gene editing 2.0: towards greater safety, efficiency and versatility.

Research Projects. List the five (5) more recent and other five (5) selected (max total 10)

Ref. Number	Date	Title	Funded by	Project Role*
1	2022	COST Action CA21113 (GenE-HumDi)	COST Action	Management Committee Member
2	2021	VALID_GT4U (EXCELLENCE/0421/0086, PI: Petros Patsali)	Research and Innovation Foundation of Cyprus	Supervisor, Research team member
3	2020	New Infrastructure for the Diagnosis and Treatment of Patients	Norway Grant 2014-2021	Grant author, scientific coordinator
4	2019	βThal-GT4U (EXCELLENCE/1216/0092)	Research and Innovation Foundation of Cyprus	PI



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5	2019	ITHANET (EXCELLENCE/1216/0256)	Research and Innovation Foundation of Cyprus	Research team member
6	2019	Advanced gene therapy delivery and models for beta-haemoglobinopathies	Telethon of Cyprus	PI
7	2017	Genome-editing-mediated lineage-specific knockdown of disease modifiers as therapy of β -globinopathies	Eurobank	PI
8	2014	Genome editing by non-homologous end joining as high-efficiency gene therapy for sickle-cell disease and β -thalassaemia	Telethon of Cyprus	PI
9	2010	THALAMOSS, grant agreement no. 306201	EC FP7	Co-author, research team member
10	2010	Advancing Gene Therapy Vectors	Research Promotion Foundation of Cyprus	Sole author, scientific coordinator

**Academic Consulting Services and/or Participation in Councils / Boards/ Editorial Committees.
List the five (5) more recent (Optional Entry)**

Ref. Number	Period	Organization	Title of Position or Service	Key Activities
1	2023-present	National Committee for Thalassaemia	Board Member	Strategizing for improved disease management and research of thalassaemia in Cyprus
2	2021-2023	Hellenic Society of Gene Therapy and Regenerative Medicine	Board Member	Conference organization and forging ties with other organizations in Greece and Cyprus



3	2015 – present	Cyprus Society of Human Genetics	Board Member, Web Content Developer (2020-2022: President)	Organisation of meetings and lectures, evaluation of submissions
4	2015	Global Globin 2020 Challenge / Global Globin Network	Executive Board Member	Organisation of meetings, data curation, consultation on laboratory technology
5	2019	Frontiers in Genome Editing	Associate Editor	Proposal of Research Topics and authors, revision of article submissions

Awards / International Recognition (where applicable). List the five (5) more recent and other five (5) selected. (max total 10) (Optional Entry)

Ref. Number	Date	Title	Awarded by:
1	28/06/2023	Awardee: Petros Kountouris; CING Outstanding Contribution Award, for his outstanding contribution promoting CING internationally as information, database and data pioneer in human genetics	The Cyprus Institute of Neurology & Genetics
2	13/06/2022	Awardee: Nikoletta Y Papaioannou; Early Career Poster Award. 55 th European Society of Human Genetics Conference, Vienna, Austria. Genome editing for beta haemoglobinopathies without double-strand DNA cleavage. <u>Papaioannou NY</u> , Patsali P, Papasavva PL, Koniali L, Naisseh B, Christou S, Lederer CW, Kleanthous M.	European Society of Human Genetics
3	20/11/2021	Awardee: Panayiota Papasavva; Best Poster Award, 1 st Prize. 8 th International Multithematic Bio-Medical Congress, Nicosia, Cyprus. Exploring miRNAs as tools for therapy: Tag-activated microRNA-mediated endogene deactivation. <u>Papasavva PL</u> , Patsali P, Loucari C, Koniali L, Papaioannou NY, Naisseh B, Kleanthous Marina, Lederer CW.	Multithematic Bio-Medical Congress
4	05/12/2020	Awardee: Panayiota Papasavva; Best Selected Abstract, 1 st Prize. 8 th International Conference of the Cyprus Society of Human Genetics, Nicosia,	Cyprus Society of Human Genetics



		Cyprus. <u>Papasavva P</u> , Papaioannou, NY, Patsali, P; Sitarou, M; Christou, S; Lederer CW [†] , Kleanthous M [†] . <i>Characterisation of miRNA expression during erythropoiesis.</i>	
5	24/09/2020	Awardee: Petros Patsali; Panos Ioannou Award, for work concerning mutation-specific gene therapy for HBIVSI-110(G>A) thalassaemia. Patsalis P, Papasavva P, Stephanou C, Turchiano G, Romito M, Christou S, Sitarou M, Mussolino C, Cornu TI, Antoniou MN, <u>Lederer CW</u> , Cathomen T, Kleanthous M. <i>Getting personal: mutation-specific gene editing and addition strategies for β-thalassemia and beyond.</i> Selected Abstract: Poster and Oral Presentation.	Cyprus Institute of Neurology and Genetics Panos Ioannou Award Selection Committee
6	07/11/2019	Awardee: Carsten W. Lederer; Best Poster Award, 1 st Prize. 7 th International Multithematic Bio-Medical Congress, Nicosia, Cyprus. <u>Lederer CW</u> , Patsalis P, Papasavva P, Stephanou C, Turchiano G, Romito M, Christou S, Sitarou M, Mussolino C, Cornu TI, Antoniou MN, Cathomen T, Kleanthous M. <i>Getting personal: mutation-specific gene editing and addition strategies for β-thalassemia and beyond.</i> Selected Abstract: Poster and Oral Presentation.	Multithematic Bio-Medical Congress
7	15/04/2019	Awardee: Petros Patsali; ESHG/CSHG National Fellowship Award for conference attendance. Young Researcher Prize candidate. Talk title: Safe and efficient personalised TALEN- and CRISPR/Cas9-based gene correction therapy for β -thalassaemia by non-viral delivery to primary cells	Cyprus Society of Human Genetics & European Society of Human Genetics
8	17/11/2018	Awardee: Panayiota Papasavva; Best Poster Award, 3 rd Prize. 6 th International Multithematic Bio-Medical Congress, Nicosia, Cyprus. <u>Papasavva P</u> , Patsali P, Loucari C, Obaid M, Stais E, Lederer CW [†] , Kleanthous M [†] . <i>CRISPR/Cas9-based disruption of beta-thalassaemia disease modifiers.</i> Poster.	Multithematic Bio-Medical Congress
9	02/06/2018	George Stamatoyiannopoulos Prize for best original research. 3 rd Congress of the Hellenic Society for Gene Therapy and Regenerative Medicine, Thessaloniki, Greece. <u>Patsali P</u> , Papasavva P, Romito M, Turchiano G, Loucari	Hellenic Society of Gene Therapy and Regenerative Medicine



		C, Stephanou C, Christou S, Sitarou M, Cornu TI, Mussolino C, Antoniou MN, Cathomen T, Lederer CW, Kleanthous M. <i>CRISPR/Cas9 and TALEN-mediated disruption of aberrant splice site restores normal gene function</i> . Selected Abstract: Oral Presentation.	
10	15/04/2017	Awardee: Petros Patsali; ESHG/CSHG National Fellowship Award for conference attendance. Young Researcher Prize candidate. Talk title: Less means more: knockdown of aberrant HBBIVSI-110(G>A) mRNA restores HBB expression and enhances gene therapy by gene addition in primary erythroid cells	Cyprus Society of Human Genetics & European Society of Human Genetics



Other Achievements. List the five (5) more recent and other five (5) selected. (max total 10) (Optional Entry)			
Ref. Number	Date	Title	Key Activities:
1	12/01/2016	Certificate "Instructor for Professional Training (Level 3)" (Εκπαιδευτής Επαγγελματικής Κατάρτισης (Επίπεδο 3)) by the government authority for human resource development Αρχή Ανάπτυξης Ανθρώπινου Δυναμικού Κύπρου (HRDA)	Participation in training and successful examination
2	28/09/2009– present	Organiser and regular presenter at the fortnightly CING Journal Club (now: <i>CING Lectures</i> site seminar), with over 150 events to date	Scheduling, contacting of speakers, organisation of the event
3	15/08/2013– present	Coordinator and Teaching Faculty; IMBS – <i>Introduction to Molecular Biomedical Sciences</i>	Course design, teaching, composition and marking of examinations
4	01/09/2012– present	Teaching Faculty (from 2023: Coordinator); MM101 – <i>Molecular Basis of Monogenic Diseases</i>	Contribution to course design, teaching, composition and marking of examinations
5	15/09/2002– present	Supervisor of four postdoctoral researchers, student supervision of six PhD students, nine MSc students and over 25 summer interns.	Supervision, thesis correction, coaching