Image courtesy of M. Cozzolino

AriSLA
MEETING 2022

Research, development and innovation in ALS

Milan

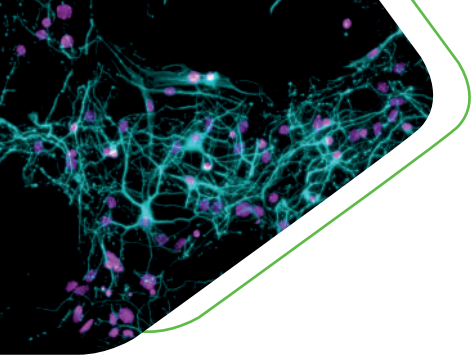
The Westin Palace

3-4 November 2022

PROGRAMME

AriSLA ETS

FONDAZIONE ITALIANA DI RICERCA PER LA SLA
SCLEROSI LATERALE AMIOTROFICA
ENTE DEL TERZO SETTORE



ARISLA ETS
FONDAZIONE ITALIANA DI RICERCA PER LA SLA
SCLEROSI LATERALE AMIOTROFICA
ENTE DEL TERZO SETTORE

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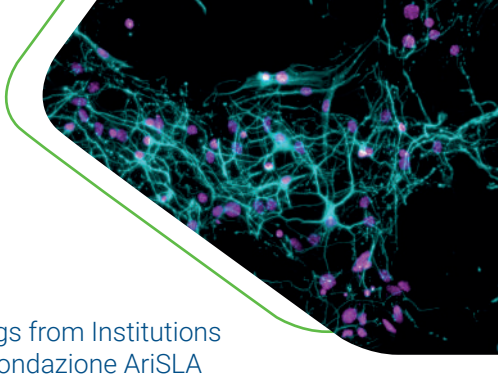
Welcome to the
2022 AriSLA Meeting!

This year's Meeting is of significant value for us, as it will be in presence after two years of the Covid pandemic.
We are very happy to renew this appointment with scientists to discuss updates on scientific progress and to offer an opportunity especially for younger people to learn and to share their research.
The program 2022 is full of guests and Lectio Magistralis by world-renowned experts, to review the state of research from basic to clinical.
At the same time, we want to promote an active discussion on which are today the most promising avenues to provide patients with tangible answers to their needs.
The message we are launching with these two days is that of concrete research, keeping the focus on people with ALS to be ever closer to new results and goals.
Thanks to all those who participate and contribute to the realization of the Meeting. Together, sharing the commitment to support research, we pursue the goal of new therapeutic tools for ALS in the near future.

Mario Melazzini
President of Fondazione AriSLA



Thursday
3rd November

08.30 - 09.00 Registration

09.00 - 09.45 Opening remarks and Greetings from Institutions
Mario Melazzini - President Fondazione AriSLA
Institution Representatives

09.45 - 10.15 **Lectio Magistralis - Luc Dupuis**
Dysregulation of energy homeostasis in amyotrophic lateral sclerosis

Session I
Pathological mechanisms in ALS - Oral presentations

Chairs: **Marco Baralle, Manuela Basso**

10.15 - 10.35 **Fabrizio D'Adda di Fagagna**
Call 2016 - DDRNA&ALS, A Role for DNA damage response RNA (DDRNA) in neurodegeneration in ALS

10.35 - 10.55 **Serena Carra**
Call 2018 - MLOpathy, Membrane-less organelle pathology in ALS: identification of causes and rescuing factors

10.55 - 11.05 General discussion

11.05 - 11.35 Coffee break

11.35 - 11.55 **Emanuele Buratti**
Call 2018 - PathensTDP, Defining the role of hnRNP proteins in enhancing TDP-43 pathology

11.55 - 12.15 **Fabrizio Chiti**
Call 2017 - TDP-43-STRUCT, Purification and determination of the structure, phase separation and toxicity of TDP-43

12.15 - 12.25 General discussion

12.25 - 14.00 Lunch

Session II
Identification of novel therapeutic targets - Oral presentations

Chairs: **Valentina Bonetto, Nicola Ticozzi**

14.00 - 14.20 **Mauro Cozzolino**
Call 2018 - SPLICEALS, Dissecting the functional interaction between FUS and hnRNP A2/B1 in the pathogenesis of ALS

14.20 - 14.40 **Alessandro Provenzani**
Call 2018 - Target-RAN, Targeting RAN translation in ALS

14.40 - 15.00 **Alberto Ferri**
Call 2017 - HyperALS, Modulation of hypermetabolism and hyperexcitability as a strategy to counteract degeneration in ALS

15.00 - 15.10 General discussion

15.10 - 15.40 Coffee break

15.40 - 16.00 **Gian Giacomo Consalez**
Call 2017 - AxRibALS, Axonal translatome and functional alterations in cellular models of amyotrophic lateral sclerosis

16.00 - 16.20 **Caterina Bendotti**
Call 2019 - MUSALS-AChR, Studying acetylcholine receptors and muscle regeneration in ALS to develop prognostic markers and potential therapies hampering disease progression

16.20 - 16.40 **Jessica Mandrioli**
Call 2015 - RAP-ALS, Rapamycin (sirolimus) treatment for amyotrophic lateral sclerosis

16.40 - 16.50 General discussion

Session III
Poster presentation

17.00 - 18.30 Poster session - Wine and cheese

Friday
4th November

Session IV
Clinical research and emerging innovative trials

Chairs: **Anna Ambrosini, Mario Melazzini**

09.00 - 09.30 **Lectio Magistralis - Lucie Bruijn**
Clinical Trial design advances in ALS

09.30 - 10.00 **Paola Marcon**
The Biogen roadmap for a diversified and synergic pipeline

10.00 - 10.45 Round Table - **Anna Ambrosini meets the AriSLA Scientific Advisory Board: Stanley H. Appel, Lucie Bruijn, Brian Dickie, Piera Pasinelli, David Taylor**
How to feed the pipeline of therapeutic approaches from preclinical to translational research

10.45 - 11.15 General Discussion

11.15 - 11.45 Coffee break

11.45 - 12.30 **Fabio Mazzeo interviews Mario Melazzini**
Institutions, companies and non profit organizations:
an alliance for health
"From experience, a perspective of research and development"

12.30 - 13.00 "2022 AriSLA Call": presentation funded project

Poster Awards "Giovani per la Ricerca"

Conclusions and closure

Speakers and Chairs
of the Meeting

Ambrosini Anna	Chief scientific officer Fondazione AriSLA, Milan
Appel Stanley H.	Johnson Center for Cellular Therapeutics, Stanley H. Appel Department of Neurology, Houston Methodist Neurological Institute, Houston (TX, USA); Comitato di indirizzo scientifico AriSLA
Baralle Marco	International Centre for Genetic Engineering and Biotechnology (ICGEB), Trieste
Basso Manuela	Department of Cellular, Computational and Integrative Biology - CIBIO, Università degli Studi di Trento
Bendotti Caterina	Istituto di Ricerche Farmacologiche Mario Negri IRCCS, Milan
Bonetto Valentina	Istituto di Ricerche Farmacologiche Mario Negri IRCCS, Milan
Bruijn Lucie	Therapeutic Area Lead, NIBR, Novartis, Basel (CH), AriSLA Scientific Advisory Board
Buratti Emanuele	International Centre for Genetic Engineering and Biotechnology (ICGEB), Trieste
Carra Serena	Università degli Studi di Modena e Reggio Emilia, Modena
Chiti Fabrizio	Dipartimento di Scienze Biomediche Sperimentali e Cliniche "Mario Serio", Università degli Studi di Firenze
Consalez Gian Giacomo	Università Vita-Salute San Raffaele, Milan
Cozzolino Mauro	Istituto di Farmacologia Traslazionale, Consiglio Nazionale delle Ricerche, Rome
D'Adda di Fagagna Fabrizio	IFOM, Istituto FIRC di Oncologia Molecolare, Milan
Dickie Brian	MND Association, Northampton (UK); AriSLA Scientific Advisory Board
Dupuis Luc	Inserm, Université de Strasbourg, UMRS-1118, Strasbourg (France)
Ferri Alberto	Istituto di Farmacologia Traslazione IFT-CNR, Roma IRCCS Fondazione Santa Lucia, Rome
Mandrioli Jessica	Dipartimento di Scienze Metaboliche, Biomediche e Neuroscienze Università degli Studi di Modena e Reggio Emilia, Modena
Marcon Paola	Principal Clinical Country and Sites Lead - Italy&Israel; Sub-Regional Lead at Biogen, Milan
Masciocchi Paolo	General Secretariat Fondazione AriSLA, Milan
Mazzeo Fabio	Scientific journalist
Melazzini Mario	President Fondazione AriSLA, Milan
Pasinelli Piera	Weinberg ALS Center, Frances & Joseph Weinberg, Vickie & Jack Farber Institute for Neuroscience, Department of Neuroscience, Thomas Jefferson University, Philadelphia, (PA, USA) and The Robert Packard Center for ALS Research, Baltimore, (MD, USA); AriSLA Scientific Advisory Board
Provenzani Alessandro	Department of Cellular, Computational and Integrative Biology - CIBIO, Università degli Studi di Trento
Taylor David	ALS Society of Canada, Toronto (CA); AriSLA Scientific Advisory Board
Ticozzi Nicola	IRCCS Istituto Auxologico Italiano, Dipartimento di Fisiopatologia Medico-Chirurgica e dei Trapianti, Centro "Dino Ferrari", Università degli Studi di Milano

Posters

P1 Defining motor cortical patterns of upper motor neuron pathology in Amyotrophic Lateral Sclerosis using a 3T-MRI with iron-sensitive sequences
Donatelli G., Costagli M., Cecchi P., Bianchi F., Migaleddu G., Becattini L., Fontanelli L., Frumento P., Siciliano G., Cosottini M.

P2 EVTestInALS - Extracellular vesicles in ALS: testing their use as biomarkers for prognosis and disease progression
Donini L., Tomè G., Migazzi A., Peroni D. , Pasetto L., Fioretti P. V., Belli R., Bonetto V., Tebaldi T., Basso M.

P3 ALS Community Support Assessment to Improve Online Access to Information and Resources in Italy
Zicchieri A.D., De Rossi N., Desiderato A., Conte Silverio F., Peviani M., Consonni M., Pozzi S., Petrozziello T., Sproviero D., De Marchi F., Grossini E., Ayala C. J.

P4 A Gene therapy Approach Targeting TDP-43 pathology for ALS
Pasetto L., Columbro S., Scozzari S., Basso M., Bonetto V.

P5 AZYGOS 2.0, Autozygosity mapping followed by next-generation sequencing in unrelated consanguineous individuals to identify novel ALS-associated genes
Brusati A., Grassano M., Calvo A., Ticozzi N.

P6 Boosting nerve REgeneration in ALS by tArgeTing the peripHery_BREATH
D'Este G., Negro S., Fabris F., Zanetti G., Megighian A., Bertoli A., Massimino M. L., Nardo G., Bendotti C., Basso M., Bonetto V., Corona C., Montecucco C., Pirazzini M., Rigoni M.

P7 Effects of therapeutic hypothermia in an animal model of Amyotrophic Lateral Sclerosis
Columbro S., Pasetto L., Dominici M., Fesce E., Elezgarai S., Biasini E., Bonetto V.

P8 Human in vitro models of TDP-43 proteinopathy for drug screening approaches
Casiraghi V., Colombrita C., Santangelo S., Invernizzi S., Sorce M. N., Silani V., Ratti A.

P9 Inhibition of class I histone deacetylases ameliorates TDP-43 pathology in experimental models of ALS
Scozzari S., Sammali E., Pasetto L., Columbro S. F., De Marco G., Margotta C., Tortarolo M., Bendotti C., A. Calvo, Bonetto V.

P10 Investigating basal mitophagy in ALS: from flies to human neurons
Favaro M., Ziviani E.

P11 KIF5A exon 27 splicing mutations: molecular analysis and splicing correction with modified U1 snRNAs
Vicidomini A., Bussani E., Romano G., Pagani F.

P12 Montelukast counteracts pathological GPR17 upregulation, oligodendrocyte dysfunction and delays disease progression in SOD1G93A amyotrophic lateral sclerosis female mice
Raffaele S., Nguyen N., Boccazzi M., Frumento G., Milanese M., Bonanno G., Abbracchio M. P., Bonifacino T., Fumagalli M.

P13 Natural killer cells modulate motor neuron-immune cell cross talk in models of Amyotrophic Lateral Sclerosis
Garofalo S., Cocozza G., Porzia A.,Inghilleri M., Scavizzi F., Raspa M., Aronica E., Bernardini G., Peng L., Ransohoff R. M., Santoni A., Limatola C.

P14 Niclosamide ameliorates disease progression in mice models of ALS
Milani M., Della Valle I., Rossi S, Cozzolino M., D'Ambrosi N., Apolloni S.

P15 Novel functionalized nanoparticles targeted to 18KDa translocator protein (TSPO) to track and modulate neuroinflammation in animal models of familial Amyotrophic Lateral Sclerosis

Gazzano A., Camazzola D., Spatafora M.G., Lamacchia A., Marsala A., Doria E., Sponchioni M., Auriemma R., Lasciafari A., Moscatelli D.,Filibian M., Peviani M.

P16 Novel insights on the role and therapeutic potential of Glycoprotein nonmetastatic melanoma protein B (Gpnmb) in Amyotrophic Lateral Sclerosis

Spatafora M. G., Cabras P., Di Nolfi G., Gazzano A., Bandirali L., Custode B. M., Dimartino A., Curti D., Biffi A., Domi T., Riva N., Peviani M.

P17 SWITCHALS - Therapeutic correction of alternative splicing defects in hnRNP A2/B1 as a way to counteract Amyotrophic Lateral Sclerosis associated with FUS

Rossi S., Milani M., Della Valle I., Bisegna S., Apolloni S., D'Ambrosi N., Cozzolino M.

P18 Targeting upper motor neurons: a novel neural stem cell therapy for Amyotrophic Lateral Sclerosis

Ongaro J., Quetti L., Ottoboni L., Contardo M., De Gioia R., Melzi V., Brambilla L., Abati E., Rizzo F., Nizzardo M., Corti S.

P19 The p97-Nploc4 ATPase complex plays a role in muscle atrophy during cancer and amyotrophic lateral sclerosis

D. Re Cecconi A., Barone M., Gaspari S., Tortarolo M., Bendotti C., Porcu L., Terribile G., Piccirillo R.

P20 Therapeutic effects of retromer stabilization in Amyotrophic Lateral Sclerosis

Mariotti V., Pedrini M., Maiocchi A., Mastrangelo E., Riva N., Milani M., Marinelli L., Martino G., Seneci P., Muzio L.

P21 OMIC characterization of patient-derived spinal cord organoids to unravel new therapeutic targets in C9ORF72 form of Amyotrophic Lateral Sclerosis

Galli N., Rizzuti M., Brambilla L., Ongaro J., Nizzardo M., Corti S.

P22 Modelling ALS disease by 3D organoids culture from human- derived iPSCs

Sorce M. N., Lattuada C., Santangelo S., Podini P., Invernizzi S., Casiraghi V., Quattrini A, Silani V., Ratti A., Bossolasco P.

P23 Spinal cord organoids from sALS patients show impairment in maturation and self-organization

Bordoni M., Scarian E., Messa L., Garofalo M., Jacchetti E., Raimondi M. T., Gagliardi S., Carelli S., Cereda C., Pansarasa O.

P24 One motor neuron at the time: uncovering the molecular logics for subtype-specific disease vulnerability

Zuccaro E., Bregolin E., Banani N., Landry J., Benes V., Cacchiarelli D., Pennuto M.

P25 A knockout zebrafish line for ALS2 gene as a new in-vivo model for Juvenile Amyotrophic Lateral Sclerosis

Tesoriero C., Greco F., Ghirotto F., Gorni G., Cadoria E., Vettori A.

P26 Association with Neuronal Development and Oncogenesis implicated-lncRNAs and SOD1-G93A Amyotrophic Lateral Sclerosis pathology

Rey F., Marcuzzo S., Bonanno S., Bordoni M., Giallongo T., Malacarne C., Zuccotti G., Carelli S., Cereda C.

P27 HuD (ELAVL4) gain-of-function phenocopies a severe ALS-FUS mutation in iPSC-derived muscle-nerve co-cultures

Silvestri B., Garone M. G., Salerno D., Mochi M., De Turris V., Medici M., Rosa A.

P28 Investigation of the lncRNA ZEB1-AS1 in sporadic ALS: deregulation in neuronal differentiation and characterization of a novel disease pathway

Maghraby E., Rey F., Messa L., Esposito L., Barzaghini B., Pandini C., Bordoni M., Gagliardi S., Diamanti L., Raimondi M. T., Mazza M., Zuccotti G., Carelli S., Cereda C.

P29 Oxygen sensing in amyotrophic lateral sclerosis: current mechanisms, implication of transcriptional response and pharmacological modulation
Messa L., Rey F., Maghraby E., Casili G.a, Ottolenghi S., Cuzzocrea S., Zuccotti G., Esposito E., Cereda C., Carelli S.

P30 Transcriptomic characterization of ALS phenotypes highlights patient-specific gene expression patterns
Garofalo M., Scarian E., Dragoni F., Di Gerlando R., Grieco L., Busacca M., Fiamingo G., Garau J., Diamanti L., Bordoni M., Pansarasa O., Gagliardi S.

P31 U1 snRNA as a novel RNA-based therapeutic approach to modulate C9ORF72 pathology in patient-derived iPSC-motoneurons
Santangelo S., Colombrita C., Bussani E., Invernizzi S., Sorce M. N., Casiraghi V., Lattuada C., Silani V., Bossolasco P., Pagani F., Ratti A.

P32 Improving muscle regeneration by virtue of peripheral macrophages: a therapeutic strategy for Amyotrophic Lateral Sclerosis
Margotta C., Fabbrizio P., Trolese M. C., D'agostino J., Suanno G., Bendotti C., Nardo G.

P33 Translatome profiling reveals deregulated neurovascular crosstalk in motor neuron disease
Brambilla I., De Pretis S., Badaloni A., Malpighi C., Bhat G., Bonanomi D.

P34 "Increased ADAM 10/17 activity in an animal model of ALS: rationale for targeting ADAMs as potential therapeutic target?"
Cabras P., Spatafora M. G., Dimartino A., Gazzano A., Peviani M.

P35 DNA methylation and histone post-translational modifications of TARDBP and subsequent modulation of TDP-43
Abou A. L., Pacetti M., De Conti L., Frascolla I., Marasco L., Romano M., Rashid M. M., Nubiè M., Baralle F., Baralle M.

P36 Senescent astrocytes drive neurodegeneration via extracellular vesicles in ALS-FTD.
Fioretti P. V., Tomè G., Soldano A., Donini L., Barbieri A., Pasetto L., Fiordaliso F., Peroni D., Belli R., D'Agostino V., Canarutto G., Piazza S., Pennuto M., Bonetto V., Tebaldi T., Basso M., Migazzi A.

P37 Large and small extracellular vesicles may contribute to the propagation of ALS and FTD carrying toxic TDP species and potentially harmful miRNAs
Casarotto E., Garofalo M., Messa L., Sproviero D., Carelli S., Cozzi M., Chierichetti M., Cristofani R., Galbiati M., Ferrari V., Piccolella M., Rusmini P., Tedesco B., Pramaggiore P., Cereda C., Gagliardi S., Poletti A., Crippa V.

P38 The small extracellular vesicles released by motoneuron mSOD1-NSC-34 cells, in vitro model of Amyotrophic Lateral Sclerosis, induce the activation of the peripheral immune system
Carata E., Muci M., Mariano S., Nigro A., Romano A., Panzarini E.

P39 Phase Separation of Full Length TAR DNA-Binding Protein (TDP-43)
Staderini T., Bigi A., Mongiello D., Chiti F.

P40 Towards unveiling the nexus between axonal granules and polysomes in ALS
Lauria F., Maniscalco F., Marchioretto M., Busarello E., Cella F., Tebaldi T., Pisciottani A., Croci L., Perrucci C., Lorenzo L., D'Antoni M., Peroni D., Basso M., Quattrone A., Siciliano V., Consalez G. G., Viero G.

P41 Sumoylation regulates tdp-43 splicing activity and nucleocytoplasmic distribution
Feligioni M., Ratti A.

P42 Circulating muscle-derived miR-206 links skeletal muscle dysfunction to heart sympathetic denervation
Ronfini M.

P43 Sympathetic neurons are additional cell types affected in Amyotrophic Lateral Sclerosis
Mazzaro A., Casola I., Vita V., Klein A., Gobbo G., Dobrowolny G., Sorarù G., Musarò A., Mongillo M., Zaglia T.

AriSLA Meeting 2022 receives the award of the

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Thanks to the Institutions, organizations and companies
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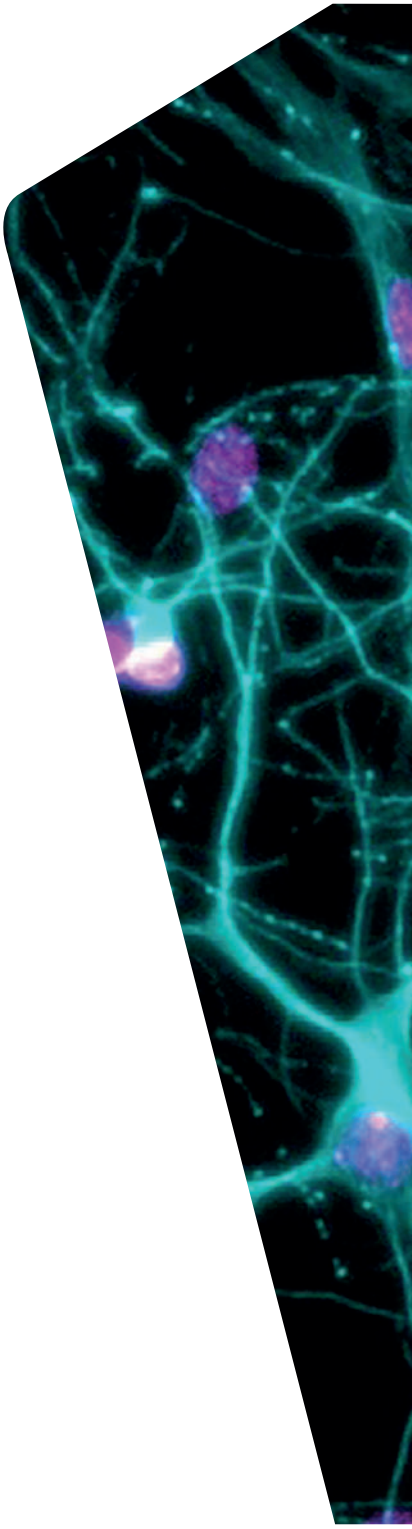

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