



# "2<sup>nd</sup> biennial conference on TDP-43 function and dysfunction in disease"

9-11 September 2025 | Trieste, ITALY



## PROGRAMME

### Monday September 8<sup>th</sup>

16.00-18.00 REGISTRATION  
Tivoli Portopiccolo Sistiana Congress Center

### Tuesday September 9<sup>th</sup>

8.30 – 9.30 REGISTRATION  
Tivoli Portopiccolo Sistiana Congress Center

9.30-9.40 Welcome address by **Emanuele Buratti**, ICGEB, Trieste, Italy

#### Session 1

### Biology, Risk & Biomarkers

Moderator Sami Barmada

9.40-10.00	The role of TDP-43 dysfunction in disease	<b>Leonard Petrucelli</b> , Mayo Clinic, USA
10.00-10.20	Uncovering environmental risk factors that drive TDP-43 proteinopathy	<b>Todd Cohen</b> , University of North Carolina, USA
10.20-10.40	Investigating the influence of UNC13A cryptic exons rs12973192 single nucleotide polymorphism on cognitive function and survival in TDP-43 proteinopathies	<b>Mercedes Prudencio</b> , Mayo Clinic, USA
10.40-10.55	TDP-43 expression is increased by ALS disease-linked TARDBP non-coding variants	<b>Vaishnavi Manohar</b> , King's College London, UK
10.55-11.10	TDP-43 PET Tracer for Imaging Aggregated TDP-43 in Neurodegenerative Diseases	<b>Elodie Chevalier</b> , AC Immune SA, Lausanne, Switzerland
11.10-11.25	<i>Discussion / Q&amp;A</i>	

#### Session 2

### Cryptic Exons & Splicing Dysregulation

Moderator Christopher Donnelly

11.25-11.45	New lessons from cryptic splicing	<b>Pietro Fratta</b> , University College London, UK
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11.45-12.05	Integrative network analysis reveals disruptive TDP-43-related cryptic splicing events related to ALS/FTD	<b>Sarah Kargbo-Hill</b> , University of Michigan, USA
12.05-12.25	Nonsense-mediated decay masks cryptic exon inclusion events caused by TDP-43 loss	<b>Aaron Gitler</b> , Stanford University, USA
12.25-12.40	Mapping Splicing Dysregulation Driven by TDP-43 in ALS	<b>Thea Meimoun</b> , Weizmann Institute of Science, Israel
12.40-12.55	Single-nucleus RNA-seq of FTLD-TDP patients reveals neuronal specificity of TDP-43 associated cryptic splicing and polyadenylation	<b>Jack Humphrey</b> , Icahn School of Medicine at Mount Sinai, USA
12.55-13.10	<i>Discussion / Q&amp;A</i>	
13.10-14.10	<i>Lunch</i>	

### Session 3

## Mechanisms of aggregate seeding and propagation

Moderator Emanuele Buratti

14.10-14.30	Muscle derived TDP-43 aggregates have prion-like properties	<b>Chris Wehl</b> , Washington University in St Louis, USA
14.30-14.50	Decoding the mechanisms linking TDP-43 aggregation to loss of function	<b>Yuna Ayala</b> , Saint Louis University, MO, USA
14.50-15.05	Spatiotemporal Spreading of Pathological TDP-43 from the Motor Cortex in the CamKIIa-hTDP-43NLSm Mouse Model of FTLD/ALS	<b>Sílvia Porta</b> , University of Pennsylvania, USA
15.05-15.20	Unraveling compartment-specific disease mechanisms using human models of TDP-43 proteinopathy	<b>Bilal Khalil</b> , VIB-KU Leuven CBD, Belgium
15.20-15.35	<i>Discussion / Q&amp;A</i>	

### Session 4

## Phase Separation & Condensates

Moderator Yuna Ayala

15.35-15.55	Modulating neuronal TDP-43 ribonucleoprotein condensates and nuclear function	<b>Pallavi Gopal</b> , Yale University, USA
15.55-16.15	Domain swapping reveals structure/function determinants	<b>Jonathan Ling</b> , Johns Hopkins University, USA
16.15-16.30	Direct binding of TDP-43 and Tau drives their co-condensation, but suppresses Tau fibril formation and seeding	<b>Francesca Simonetti</b> , DZNE – Munich, Germany
16.30-16.45	Is there a role for the coronavirus nucleocapsid (N) protein in pathological TDP-43 biomolecular condensate formation?	<b>Michael Strong</b> , Western University, Canada
16.45-17.00	Phase separation of TDP-43 and its fragments in vitro and in cells	<b>Fabrizio Chiti</b> , University of Florence, Italy
17.00-17.15	<i>Discussion / Q&amp;A</i>	
17.15-17.35	<i>Coffee break</i>	

Session 5  
**Structural Insights**  
 Moderator Edward Lee

17.35-17.55	Structures of pathological TDP-43 in neurodegenerative diseases	<b>Benjamin Ryskeldi-Falcon</b> , MRC Laboratory of Molecular Biology, UK
17.55-18.15	Molecular Visualization of Neuronal TDP43 Pathology In Situ	<b>Shyamal Mosalaganti</b> , University of Michigan, USA
18.15-18.30	Replacement of TDP-43's prion like domain does not compromise localization, function or viability in vivo	<b>Aarti Sharma</b> , Motor Neuron Diseases, USA
18.30-18.45	<i>Discussion / Q&amp;A</i>	
18.45	<i>Group photo</i>	
19.00	<i>Welcome reception</i>	

**Wednesday September 10<sup>th</sup>**

Session 6  
**Pathology & Mechanisms**  
 Moderator Sarah Maria Mizielinska

9.00-9.20	Using single molecule, biophysical and cell approaches to unveil structure-function alterations to the nuclear pore in C9ORF72 TDP-43 proteinopathy	<b>Sarah Marie Mizielinska</b> , King's College London, UK
9.20-9.40	TDP-43 at the synapse	<b>Chris Henstridge</b> , University of Dundee, UK
9.40-10.00	Role of membrane trafficking in TDP-43 aggregation	<b>Masahisa Katsuno</b> , University of Nagoya, Japan
10.00-10.15	PIAS-4 mediated SUMO2/3-ylation of TDP-43 protects against aggregation	<b>Serena Carra</b> , University of Modena and Reggio Emilia, Italy
10.15-10.30	Lysosomal disruptions in neurons with TDP-43 pathology in ALS	<b>Adam Walker</b> , University of Sydney, Australia
10.30-10.45	<i>Discussion / Q&amp;A</i>	
10.45-11.05	<i>Coffee break</i>	

Session 7  
**Mechanisms of Dysfunction**  
 Moderator Jemeen Sreedharan

11.05-11.25	Annexin A11 Proteinopathies: Implications for Pathologic Subtyping of TDP-43 Proteinopathies	<b>Edward Lee</b> , University of Pennsylvania, USA
11.25-11.45	TDP-43 interactions the modulate function and dysfunction in neurological disease	<b>Christopher Donnelly</b> , University of Pittsburgh School of Medicine, USA
11.45-12.00	Emerging role of Citrullination as a novel post-translational modification of TDP-43 implicated in the neuropathology of LATE and Alzheimer's Disease Related Dementia	<b>Maj-Linda Selenica</b> , Sanders-Brown Center on Aging, USA

12.00-12.15	Optineurin insufficiency ameliorates cognitive deficits and lipopolysaccharide-induced sickness behavior in the TDP-43G348C mouse model of frontotemporal dementia	<b>Josip Peradinovic</b> , Faculty of Biotechnology and Drug Dev., Croatia
12.15-12.30	In situ proteomic profiling of TDP-43 aggregates in human FTLD-TDP brain tissue	<b>Wilfried Rossoll</b> , Mayo Clinic Florida, USA
12.30-12.45	<i>Discussion / Q&amp;A</i>	
12.45-13.45	<i>Lunch</i>	

### Session 8

## RNA Control & Metabolism

Moderator Michael Ward

13.45-14.05	A toolbox for modelling nuclear loss and cytoplasmic gain of TDP-43 function	<b>Marc-David Ruepp</b> , King's College London, UK
14.05-14.25	Regulation and impact of alternatively spliced TDP43 isoforms	<b>Sami Barmada</b> , University of Michigan School of Medicine, Ann Arbor, MI, USA
14.25-14.45	TDP-43 governs the global control of the m6A epitranscriptome	<b>Jobert Vargas</b> , University College London, UK
14.45-15.00	TDP-43 Dysfunction Compromises UPF1-Dependent mRNA Metabolism Pathways	<b>Francesco Alessandrini</b> , Northwestern University, USA
15.00-15.15	<i>Discussion / Q&amp;A</i>	
15.15-15.35	<i>Coffee break</i>	

### Session 9

## Network & High-Throughput Screens

Moderator Wilfried Rossoll

15.35-15.55	TDP-43 Import: A Whole genome CRISPRi screen in human iPS neurons reveals multiple ALS, FTD, and ALS genes that that alter TDP-43 import	<b>Jeffrey Rothstein</b> , Johns Hopkins University, USA
15.55-16.15	CRISPRi screening identifies modifiers of TDP-43 loss-of-function-mediated neurotoxicity	<b>Shuying Sun</b> , Johns Hopkins University, USA
16.15-16.35	Next-gen screens of TDP-43 biology in iPSC-derived neurons	<b>Michael Ward</b> , NIH / NINDS, USA
16.35-16.50	Integrative network analysis links TDP-43-driven splicing defects to cascading proteomic disruption of ALS/FTD-associated pathways	<b>Velina Kozareva</b> , Massachusetts Institute of Technology, USA
16.50-17.05	RGNEF N-terminal fragment mitigates TDP-43 toxicity in ALS: Role of the LeuR domain in its biological activity and insights from spatial transcriptomics	<b>Cristian Droppelmann</b> , Robarts Research, Western University, Canada
17.05-17.20	<i>Discussion / Q&amp;A</i>	
17.20-20.00	<i>Free time</i>	
20.00-22.00	<i>Poster session with aperitif</i>	

## Thursday September 11<sup>th</sup>

8.50-9.00 *Winners of poster session announcement*

### Session 10

#### Therapeutic Strategies

Moderator Adam Walker

9.00-9.20	Short RNA chaperones promote aggregation-resistant TDP-43 conformers to mitigate neurodegeneration	<b>James Shorter</b> , University of Pennsylvania, USA
9.20-9.40	TDP-43 in Disease: From Structural Biology to Therapeutic Innovation	<b>May Khanna</b> , University of Florida, USA
9.40-9.55	Targeting TDP-43 in ALS: Preclinical and Translational Development of AP-2, a CK-1 $\delta$ Inhibitor	<b>Ana Martinez</b> , Centro Investigaciones Biologicas CSIC, Spain
9.55-10.10	Small molecule modulator of importin- $\beta$ 1 prevents and reverses TDP-43 Aggregation In-vitro and In-vivo	<b>Marc Shenouda</b> , University of Toronto, Canada
10.10-10.25	New technologies for ALS/FTD gene therapies	<b>Oscar Wilkins</b> , UCL, UK
10.25-10.40	<i>Discussion / Q&amp;A</i>	
10.40-11.00	<i>Coffee break</i>	

### Session 11

#### Translational & Disease Signatures

Moderator Jenna Gregory

11.00-11.20	Distinct pathological TDP-43 signatures exist across ageing, amyotrophic lateral sclerosis and Alzheimer's disease	<b>Jenna Gregory</b> , University of Aberdeen, UK
11.20-11.40	Profiling individual TDP-43 aggregates reveals a new molecular signature of Motor Neuron Disease	<b>Dezerae Cox</b> , University of Wollongong, Australia
11.40-11.55	Isoform-specific SUMOylation modulates TDP-43 phase separation	<b>Philipp Schönberger</b> , Institute of Molecular Biology, Germany
11.55-12.10	Altered TDP-43 function leads to impaired cholesterol homeostasis associated with deficits in myelination	<b>Irene García Toledo</b> , IdISSC, Spain
12.10-12.25	Distinct RNA and transcriptome changes in Alzheimer's disease patients with TDP-43 pathology (AD-LATE)	<b>Nicole Liachko</b> , University of Washington/ VA Puget Sound, USA
12.25-12.40	<i>Discussion / Q&amp;A</i>	
	<i>Closing remarks</i>	

In collaboration with

