PSPA

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UNLOCKING THE SECRETS OF BRAIN DISEASE®

PSP & CBD

INTERNATIONAL RESEARCH SYMPOSIUM 2018

October 25–26, 2018

Royal College of Physicians
11 St Andrews Place
Regent's Park, London NW1 4LE, UK

Welcome to the

PSP & CBD
INTERNATIONAL RESEARCH SYMPOSIUM 2018

Welcome to the First International PSP & CBD Research Symposium. This year, the PSP Association and CurePSP joined forces in bringing the tau research community together. We are thrilled by the overwhelming response we have received not only from both sides of the Atlantic, but also from Asia, Africa, and South America. It is wonderful to see how research in PSP and CBD has grown and produced some very encouraging results, sending out a message of hope for our patients, families, and caregivers. We are looking forward to one-and-half-days of learning about cutting-edge research in the fields of PSP, CBD, and beyond. Topics range from basic to clinical research, including biomarker development, care research, and pathological mechanisms in tauopathies. We will hear about the genetics of PSP and CBD, current clinical trials, international neuropathological studies, and patient registries. Please pay extra attention to our poster sessions; many of the presenters are early-career researchers who will present very exciting results from their labs. We are delighted that Her Royal Highness The Duchess of Gloucester has kindly agreed to open this year's meeting, underlining the importance of this research for our societies. Last but not least, we would like to recognize and thank our sponsors, without whom this meeting would not have been possible.

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SYMPOSIUM SCHEDULE

THURSDAY, OCTOBER 25

Welcome Reception &

Discussion Sessions: 6:00–7:20pm

PSPA Clinical Research Fellow: 6:20–6:35pm

CurePSP Grantee: 6:35–6:50pm

Keynote Lecture: 6:50–7:20pm

Poster Presentation and Cocktail Reception: 7:20–9:00pm

FRIDAY, OCTOBER 26

Registration: 7:30–8:20am

Symposium: 8:20am–5:25pm

Poster Presentation and Cocktail Reception: 5:25–8:00pm

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2018 International Research Symposium Agenda

THURSDAY, OCTOBER 25, 2018

6:00 – 6:20pm **Welcome and Introductions**
Her Royal Highness The Duchess of Gloucester, Royal Patron, PSPA
Alex Klein, *CurePSP, New York, NY (USA)*
Kate Arkell, *PSP Association, Milton Keynes (UK)*

6:20 – 6:35pm **PSPA Clinical Research Fellow**
Chair: **James Rowe,** *University of Cambridge, Cambridge (UK)*
Developing Biomarkers for PSP
Edwin Jabbari, *UCL, London (UK)*

6:35 – 6:50pm **CurePSP Grantee**
Chair: **Alex Klein,** *CurePSP, New York, NY (USA)*
In-Home Care for PSP Patients
Jori Fleisher, *Rush University, Chicago, IL (USA)*

6:50 – 7:20pm **Keynote Lecture**
Chair: **Huw Morris,** *UCL, London (UK)*
Twenty-Five Years' Progress in Progressive Supranuclear Palsy
Lawrence I. Golbe, *Rutgers University, New Brunswick, NJ (USA)*
Irene Litvan, *University of California, San Diego, CA (USA)*

7:20 – 9:00pm **Posters and Cocktail Reception**

FRIDAY, OCTOBER 26, 2018

7:30 – 8:20am **Registration**

8:20 – 8:30am **Welcome**
John C. Steele, *Villa Cahya Pendet Museum, Ubud, Bali (Indonesia)*

8:30 – 8:55am **Keynote Lecture**
Chair: **Karen Duff,** *Columbia University, New York, NY (USA)*
Tau-Induced Laminopathy is a Mechanistic Driver of Neurodegeneration
Bess Frost, *University of Texas San Antonio (USA)*

8:55 – 9:40am **Neuropathology**
Chair: **James Rowe,** *University of Cambridge, Cambridge (UK)*
Update on the Mayo Clinic Brain Bank
Dennis Dickson, *Mayo Clinic, Jacksonville, FL (USA)*
Update on the Queen Square Brain Bank
Janice Holton, *Queen Square Brain Bank, London (UK)*
Mapping the Progression of Tauopathies
Helen Ling, *UCL, London (UK)*

9:40 – 10:00am **Coffee Break and Poster Session**

10:00am – 12:00pm **Genetics Session and Panel Discussion**
Chair: **Jeff Friedman,** *CurePSP, San Diego, CA (USA)*
Insights Into the Genetics of PSP
John Hardy, *UCL, London (UK)*
Update on the PSP Genetics Consortium
Gerard Schellenberg, *University of Pennsylvania, Philadelphia, PA (USA)*
Epigenetics of PSP
Günter U. Höglinger, *German Center of Neurodegenerative Diseases, Munich (Germany)*
Clinical and Genetic Variation in PSP
Huw Morris, *UCL, London (UK)*
Big (Omics, WGS) Data Collection in PD: A Model for PSP?
Marg Sutherland, *NINDS, Bethesda, MD (USA)*

12:00 – 1:00pm **Lunch and Poster Session**

1:00 – 1:30pm **Keynote Lecture**
Chair: **Jean-Christoph Corvol,** *Hôpital Pitié-Salpêtrière, Paris (France)*
Tauopathy—Insights from the Pathology, Pathological Mechanisms, and Therapeutic Opportunities
Karen Duff, *Columbia University, New York, NY (USA)*

1:30 – 3:00pm **Clinical Research**
Chair: **Lawrence I. Golbe,** *Rutgers University, New Brunswick, NJ (USA)*
Breaking News in PSP/CBD Clinical Trials
Adam Boxer, *University of California San Francisco, CA (USA)*
How Representative is the Population Included in PSP Clinical Trials?
Jean-Christoph Corvol, *Hôpital Pitié-Salpêtrière, Paris (France)*
Lesson Learnt from HD Trials
Anne Rosser, *Cardiff University, Wales (UK)*
Pharma Updates
Panel Discussion
Timothy Buchanan, *UCB, Braine-l'Alleud (Belgium)*
Tien Dam, *Biogen, Boston (USA)*
Jorge Zamudio, *AbbVie, Chicago, (USA)*

3:00 – 3:20pm **Coffee Break and Poster Session**

3:20 – 4:05pm **Registries/Natural History Studies**
Chair: **Huw Morris,** *UCL, London (UK)*
Update on the FTD Disorder Registry
Dianna Wheaton, *FTD Disorder Registry, Dallas, Texas (USA)*
Update on the Prospect Study and CBD Registry
John Woodside, *UCL, London (UK)*
Update on DescribePSP and ProPSP Studies
Gesine Respondek, *DZNE, Munich (Germany)*

4:05 – 5:05pm **Tau Biomarkers and Imaging**
Chair: **Günter U. Höglinger,** *DZNE, Munich (Germany)*
Latest Results in Tau-PET Imaging
James Rowe, *University of Cambridge, Cambridge (UK)*
Latest Results in Fluid Biomarkers
Henrik Zetterberg, *University of Gothenburg (Sweden)*
Latest Results in MRI Imaging
Jennifer Whitwell, *Mayo Clinic, Rochester, MN (USA)*

5:05 – 5:15pm **PSPA/CurePSP Early-Career Researcher Award + Poster Prize**

5:15 – 5:25pm **Closing Remarks**
Andrew Symons, *PSP Association, Milton Keynes (UK)*
David Kemp, *CurePSP, New York, NY (USA)*

5:25 – 8:00pm **Poster Session and Cocktail Reception**

Agenda is subject to change.

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Symposium Speakers

Kate Arkell

Kate has an honours degree in Physiological Sciences from the University of Newcastle-upon-Tyne. She spent six years as Research Information Coordinator at the Motor Neurone Disease Association, where she was involved in developing research information across a range of channels for patients and their families as well as the scientific community. Kate is now coordinating the research activity of PSPA, managing the charity's grants programme and developing its position as a facilitator of PSP and CBD research.

Adam L. Boxer

Adam L. Boxer, MD, PhD, is Endowed Professor in Memory and Aging in the Department of Neurology at the University of California, San Francisco (UCSF). He directs UCSF's Neurosciences Clinical Research Unit and the Alzheimer's Disease and Frontotemporal Degeneration (FTD) Clinical Trials Program at the UCSF Memory and Aging Center. He is the Principal Investigator of the Advancing Research and Treatment for FTLD (ARTFL) Rare Disease Clinical Research Consortium.

Tim Buchanan

Tim, Senior Director, Translational Medicine Neurology, UCB Pharma, is the Clinical Lead for the Tau programme at UCB and is based in Belgium. He has been with UCB New Medicines since 2007. Prior to that Tim worked for Pfizer in the UK for 16 years.

Jean-Christoph Carvol

Jean-Christoph Carvol, Professor of Neurology, is the head of the Clinical Research Center for Neurosciences at the ICM, Pitié-Salpêtrière Hospital in Paris, France. He has training in both clinical and basic research in neurosciences. His field of interest is pharmacology and genetics of PD and movement disorders. He is co-chair of the national clinical research network for PD and movement disorders in France (NS-PARK/FCRIN) and President of the scientific committee of the French patient organization for PSP (PSP France).

Tien Dam

Tien Dam was trained in the USA and is board certified in Internal Medicine and Geriatrics. She has 14 years' experience in academia and several years in the pharmaceutical industry. At Biogen, she is the Medical Director for the anti-tau antibody currently being investigated as a potential treatment in progressive supranuclear palsy.

Dennis W. Dickson

Dr. Dickson's professional career has been devoted to the neuropathology of degenerative disorders, particularly those that produce dementia and Parkinsonism. He is the director of the brain bank for neurodegenerative disorders at Mayo Clinic, which houses the world's largest collection of atypical parkinsonian disorders. He received his BS and MD degrees from the University of Iowa College of Medicine; his awards include the Metropolitan Life Award (2001) and Potamkin Prize (2011).

Karen Duff

Dr. Duff received her PhD from Sydney Brenner's department at the University of Cambridge (UK) in 1991. Over the last fourteen years, Dr. Duff has used genetic engineering technology to create several mouse models for AD that develop either plaques or tangles. Currently, her main interest is in exploring how tangles form in the AD brain, again using mouse models that she has created. Dr. Duff's CV includes over 100 peer reviewed research articles, and she is a regular speaker at scientific meetings around the world. Her work is funded mainly by the NIH and the Alzheimer's Association.

Jori Fleisher

Jori Fleisher, MD MSCE, is a movement disorders specialist and health services researcher focused on understanding and addressing the needs of individuals and families living with advanced Parkinson's disease, progressive supranuclear palsy, and related disorders. Dr. Fleisher has been recognized as an Emerging Leader and outstanding patient advocate by the American Academy of Neurology, and has been involved in patient education for CurePSP, Parkinson Foundation, and Michael J. Fox Foundation.

Jeff Friedman

Dr. Friedman oversees a mixed portfolio of private and public companies in the biotechnology and pharmaceutical space, with an emphasis on platform technologies, oncology, neurodegenerative disease, and genomics. He is the Chief Operating Officer of DTx Pharma, a San Diego-based company developing a novel approach to delivery of nucleic acid medicines (siRNA and antisense). He is an active investor, board member, or advisor to a number of early-stage companies in the biotechnology space, primarily in San Diego.

Bess Frost

Dr. Bess Frost is an Assistant Professor at the Barshop Institute for Longevity and Aging Studies, the Glenn Biggs Institute for Alzheimer's and Neurodegenerative Disease, and the department of Cell Systems and Anatomy at the University of Texas Health San Antonio. A current major focus of the Frost laboratory is on the consequences of tau-induced disruption of nuclear and genomic architecture in regard to RNA handling and transposable element activation.

Lawrence I. Golbe

Lawrence I. Golbe, MD, is Emeritus Professor of Neurology at Rutgers Robert Wood Johnson Medical School in New Brunswick, NJ. His research is in the clinical genetics, clinimetrics, and epidemiology of the parkinsonian disorders. In 1997, he led the clinical portion of the project that identified the first known Mendelian mutation causing Parkinson's disease in the gene for alpha-synuclein. He devised the PSP Rating Scale, which since its publication in 2007, has become the standard clinical measure and treatment outcome marker for PSP worldwide.

John Hardy

John Hardy received his degree in Biochemistry from Leeds in 1976 and his PhD from Imperial College in Neuropharmacology in 1979. He did postdocs at the MRC Neuropathogenesis Unit and the Swedish Brain Bank, in Umea, where he started to work on Alzheimer's disease. He has won the Allied Signal, Potamkin, MetLife, and Kaul Prizes for his work on Alzheimer's disease and the Anna Marie Opprecht Prize for his work on Parkinson's disease. More recently he was awarded the 2011 Khalid Iqbal Lifetime Achievement Award in Alzheimer's Disease Research and the IFRAD 2011 European Grand Prize for Alzheimer's Research. In 2014, he was awarded the Dan David Prize endowed by the Dan David Foundation, headquartered at Tel Aviv University; the Thudichum Medal from the Biochemical Society and is the recipient of the 3rd Lord Brain Memorial Medal.

Günter U. Höglinger

Professor Günter U. Höglinger, MD, heads the Institute for Translational Neurodegeneration of the German Center for Neurodegenerative Diseases (DZNE) and is Consultant Senior Neurologist at the Dept. of Neurology in the Klinikum rechts der Isar of the Technical University Munich (TUM), Germany. His present research focuses on the identification of environmental and genetic causes of neurodegenerative akinetic-rigid and dementing syndromes including PSP and CBD, the improvement of diagnostic tools, and the development of therapeutic interventions.

Symposium Speakers

Janice Holton

Janice Holton is Professor in Neuropathology at the UCL Institute of Neurology and Honorary Consultant Neuropathologist at the National Hospital for Neurology and Neurosurgery (NHNN). She gained her PhD from the University of Southampton, trained in neuropathology at the NHNN and became a Member of the Royal College of Pathologists in 1998. Janice was appointed as Director of Neuropathology at the Queen Square Brain Bank for Neurological Disorders (QSBB) in 2014. She has published over 150 peer reviewed papers in neurodegeneration and has been an invited speaker at a number of international meetings.

Edwin Jabbari

Dr. Edwin Jabbari qualified from University College London Medical School with distinction in 2011. He completed general medical training in London, including a neurology rotation at the National Hospital for Neurology and Neurosurgery. Since 2016, he has been working at the Institute of Neurology, UCL, as a PSP Association funded clinical research fellow and is also currently undertaking a PhD. His PhD research focuses on the biological determinants of disease progression in PSP.

Alex Klein

Dr. Alex Klein was appointed Vice President of Scientific Affairs at CurePSP in July 2015. He earned his PhD in neuroscience from the University of Freiburg, Germany, working in the field of restoration of motor function using stem cells in the context of Parkinson's and Huntington's. Dr. Klein also holds a Master's degree in biology from the University of Tuebingen, Germany. Dr. Klein oversees the CurePSP grant portfolio and works closely with patients and caregivers, academia, and industry to accelerate research in tauopathies.

Helen Ling

Helen is a neurologist with a special interest in movement disorders. Her main research interest is clinic-pathological correlations in tauopathies including corticobasal degeneration, progressive supranuclear palsy, and chronic traumatic encephalopathy. Helen is currently funded by CBD Solutions to study the pathological progression of CBD at Queen Square Brain Bank. Helen was awarded the prestigious Junior Award for Excellence in Clinical Research by the International Movement Disorders Society and the Charles Symonds Prize by the Association of British Neurologists.

Irene Litvan

Irene Litvan, MD, is a board-certified neurologist and director of the Parkinson and Other Movement Disorders Center at UC San Diego Health. Dr. Litvan treats and diagnoses individuals with Parkinson's disease, progressive supranuclear palsy, dementia with Lewy bodies, corticobasal degeneration, multiple system atrophy, Huntington's disease, essential tremor, and frontotemporal dementias. Dr. Litvan's research focuses on a better understanding of Parkinsonian and dementia disorders and on the search for novel therapeutic approaches including symptomatic and disease modifying therapies, as well as novel technology for better management of these diseases.

Huw Morris

Huw Morris is Professor of Clinical Neuroscience at UCL Institute of Neurology, and Honorary Consultant Neurologist at the Royal Free Hospital and the National Hospital, Queen Square. His clinical and research training took place at the National Hospital, the Mayo Clinic, and the western Pacific island of Guam. He began research in PSP and related disorders as a PSP Association and MRC Clinical Research Fellow in 1997. His main interests are neurogenetics, movement disorders, and dementia. He is leading a new UK-wide research network in PSP and corticobasal degeneration (PROSPECT), a UK-wide research project in genetic Parkinson's (Parkinson's families project), and a study of biomarkers in sports concussion.

Gesine Respondek

Gesine Respondek, MD, is a neurologist and postdoctoral fellow at the hospital of the Technical University Munich and the German Center of Neurodegenerative Diseases in Munich, Germany. She joined Professor Günter Höglinger´s team in 2007. Her current research is focused on improving the efficiency of diagnosing progressive supranuclear palsy.

Anne Rosser

Anne Rosser is Professor of Clinical Neuroscience at Cardiff University and Honorary Consultant Neurologist at the University Hospital of Wales. She trained in Medicine at Cambridge University where she also undertook her PhD in Neuroscience. She has a special interest in Huntington's disease (HD), in particular the development of new therapies for this and related neurodegenerative conditions. She directs the Cardiff University Brain Repair Group; leads the South Wales HD clinical service; co-directs the Wales Brain Repair and Intracranial Neurotherapeutics (BRAIN) Unit, and is Chair of the European HD Network.

James Rowe

James leads the Cambridge Centre for Frontotemporal Dementia and related disorders (including regional healthcare clinics for PSP & CBD), is a principal investigator in the Cambridge Centre for Parkinson Plus, and is honoured to be a Trustee of the PSP Association. His research into the pathophysiology and cognitive syndromes of PSP & CBD promotes the development and validation of disease-specific biomarkers and new therapeutic strategies. This brings together advances in PET, MRI, MEG, and pharmacological interventions to understand and restore brain function.

Gerard D. Schellenberg

Dr. Gerard D. Schellenberg is a professor in the Department of Pathology and Laboratory Medicine in the Perelman School of Medicine, University of Pennsylvania. He works on the genetics and molecular biology of a number of neurodegenerative diseases, including progressive supranuclear palsy, corticobasal degeneration, and Alzheimer's disease. He is co-director of the Genomics Center for Alzheimer's disease and co-director of the Penn Neurodegeneration Genomics Center. He has been working on PSP genetics for the past 15 years.

John Steele

John Steele is a clinical neurologist who assisted Drs. J. C. Richardson and Jerzy Olszewski of Toronto to describe progressive supranuclear palsy in 1964. With hopes of learning its cause and cure, he travelled half way around the world to investigate the Chamorros of Guam who, for two centuries had suffered a paralyzing disease similar to PSP, which they called lytico-bodig. Despite many years and many studies, Steele and his colleagues did not learn its cause, or the cause and cure of PSP, as he had hoped. And when the lytico-bodig disease ended by 2014, he left Guam to live in Bali, Indonesia. His passion to understand the mechanisms of neurodegeneration and their cure continues there.

Marg Sutherland

Dr. Sutherland completed her undergraduate degree in Microbiology and Immunology at the University of Western Ontario and her doctoral degree with Professor Eric Barnard in the Molecular Neuroscience Unit at the Laboratory of Molecular Biology (LMB) in Cambridge, UK, studying the molecular biology of GABA receptors. She completed her postdoctoral training with Dr. Jeff Noebels at the Baylor College of Medicine, where she developed transgenic mouse models of absence seizures (overexpression of voltage-gated K+ channels) and enhanced astroglial glutamate transport. Her current grant portfolio includes basic, translational, and clinical research grants on Frontotemporal Degeneration and Parkinson's disease.

Dianna K.H. Wheaton

Dr. Wheaton has more than 20 years of clinical research experience and genetic counseling in the field of rare neurodegenerative diseases, acting as investigator for clinical trials and genetic epidemiology studies, as well as authoring numerous genotype-phenotype papers. As Director of the FTD Disorders Registry, she works directly with persons diagnosed with FTD and their families, leads outreach efforts to the lay and health professional communities, and actively works with clinicians, researchers, and organizations interested in using the Registry to answer important research questions and to support clinical trials.

Jennifer Whitwell

Dr. Whitwell is a Professor of Radiology at Mayo Clinic, Rochester, MN. She has focused her research career on the investigation of neuroimaging biomarkers in different neurodegenerative disorders, including PSP and CBD. She has published over 180 peer-reviewed manuscripts, as well as over 20 reviews, book chapters, and editorials, with over 45 publications on PSP and CBD. Based on her accomplishments, she has received a number of prestigious awards, including the Alzheimer's Association New Investigator De Leon Neuroimaging Award, and she is currently PI on three R01 grants from the National Institutes of Health.

John Woodside

John Woodside graduated with honours from the University of Glasgow, followed by a PhD from the University of Glamorgan, focusing on intermittent hypoxia and redox regulation of vascular nitric oxide bioavailability and implications on cerebral and muscle oxygenation. Recently, John has been working in clinical research at UCL for 8 years, starting at the Institute of Cardiovascular Science where he worked on the AdDIT and 1946 cohorts and has been leading the PROSPECT study since its inception in 2015.

Jorge Zamudio

Dr. Jorge Zamudio completed his medical degree at the Universidad del Rosario in Bogota, Colombia. Dr. Zamudio has dedicated the last decade of his life to the research of neurodegenerative diseases, primarily movement disorders. Currently, he is the Global Medical Director for Neurosciences Medical Affairs in AbbVie and works with movement disorder specialists around the world to advance the science in PSP. Before joining AbbVie he worked for the Parkinson's Foundation (formerly National Parkinson's Foundation [NPF]) where he was the Director of Research Programs and oversaw the Foundation's Centers of Excellence Program.

Henrik Zetterberg

Henrik Zetterberg is a Professor of Neurochemistry at the University of Gothenburg, Sweden, and University College London, UK, and a Clinical Chemist at the Sahlgrenska University Hospital in Gothenburg. He is Head of the Department of Psychiatry and Neurochemistry at the University of Gothenburg, and his main research focus and clinical interest is fluid biomarkers for central nervous system diseases, neurodegenerative diseases in particular. He has published more than 900 papers and has received numerous awards.

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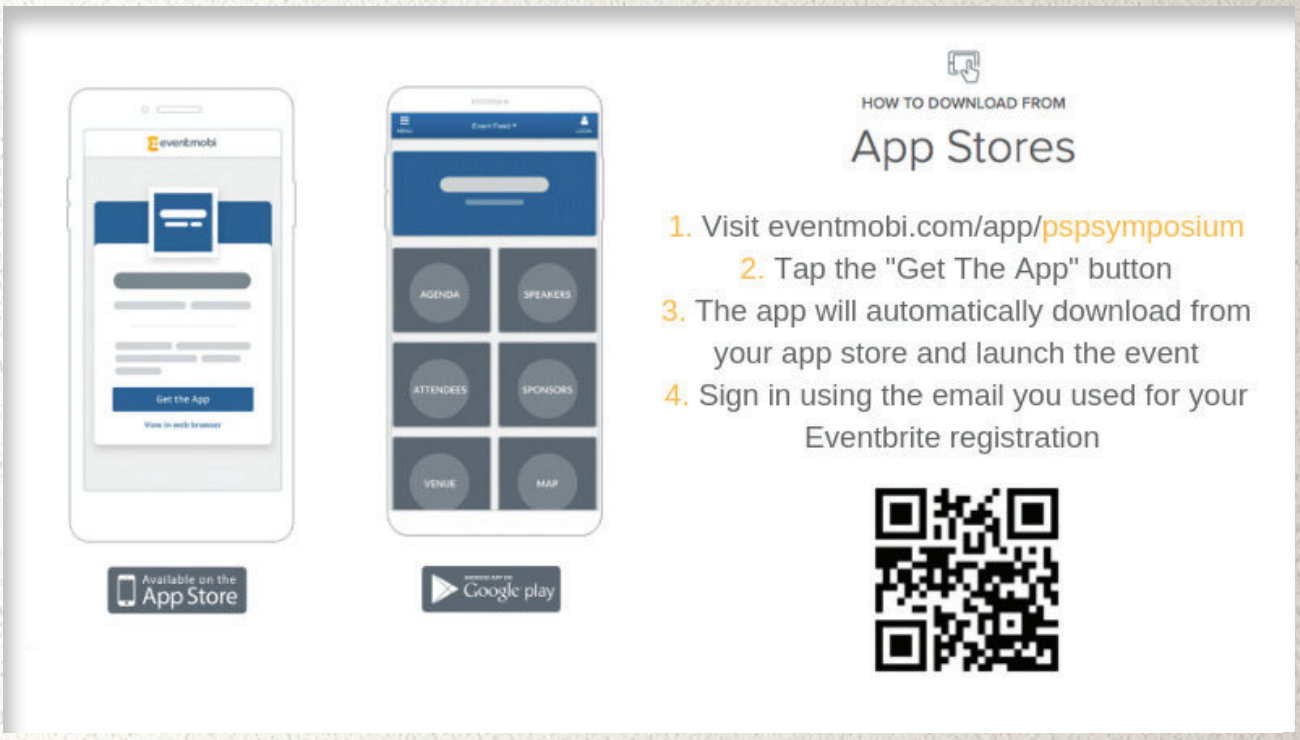

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