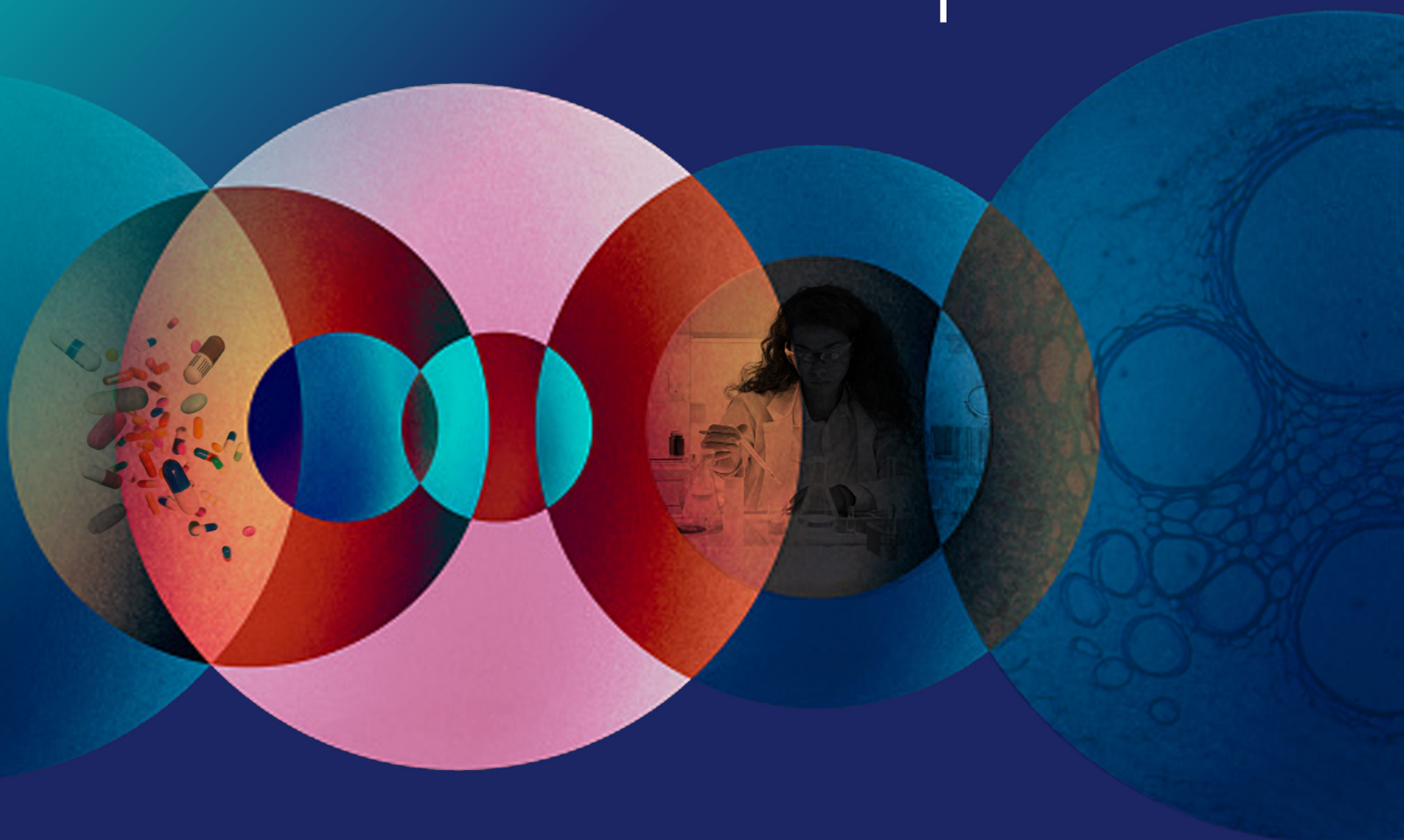


IQ MPS AFFILIATE 2020

Year-End Report



INTERNATIONAL CONSORTIUM *for*
INNOVATION & QUALITY
in PHARMACEUTICAL DEVELOPMENT

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INTRODUCTION

During 2020, IQ MPS continued to support the implementation of MPS in drug development by facilitating dialogue between industry stakeholders and regulators and providing a forum for data sharing and collaboration amongst members. The group met with FDA once in person and held its first IQ MPS-FDA webinar in what is now a well-established series. In addition to its engagement with regulatory agencies, IQ MPS established a partnership with the Predictive Safety Testing Consortium (PSTC) and expanded its reach abroad by engaging with the European Organ-on-Chip Society (EUROoCS) and the National Centre for the Replacement, Refinement, and Reduction of Animals in Research (NC3Rs). Laying the groundwork for collaborative studies to characterize MPS, IQ MPS released a RFI on Gastrointestinal MPS. Further establishing the group as a thought leader in the field, IQ MPS published the final manuscript from its first collection of Organotypic manuscripts, and subsequently formed eight new subteams to develop manuscripts on topics ranging from reproductive MPS models to the use of MPS in cell and gene therapy development.



LETTER FROM THE CHAIRS



Terry Van Vleet, PhD, DABT
Past Chair (2020-2021)
Director of Investigative
Toxicology, AbbVie



Jason Ekert, PhD, MBA
Chair (2021-2022)
Senior Director,
GlaxoSmithKline
Pharmaceutical R&D

Welcome to our 2020 Year in Review. In looking back on 2020, we are proud of all that the IQ MPS Affiliate has accomplished. Despite the intense challenges of the pandemic, the group remained focused and productive throughout the year serving as a critical thought leader for stakeholders in the implementation and qualification of MPS models. At the beginning of the year, IQ MPS hosted a highly successful in-person meeting with the FDA, delivering on a priority goal to engage with the agency on barriers to applying MPS in drug discovery and development.

The group's flexibility and perseverance throughout the year was also impressive. In place of our annual October in-person Steering Committee meeting, we pivoted to organize an engaging and productive virtual meeting. IQ MPS also fostered a spirit of community among members through team bonding activities during the difficult months of quarantine. In this way, the group maintained a much-needed source of connection with colleagues in similar situations.

With the continued support and engagement of our members, we look forward to continuing to expand the IQ MPS Affiliate network and mission. We wish everyone good health and safety in the coming year and hope for even more productivity in 2021!



2020 YEAR IN REVIEW

21

MEMBER COMPANIES

88

ACTIVE PARTICIPANTS



3

IN-PERSON AND
VIRTUAL MEETINGS
WITH REGULATORS

9

PRESENTATIONS



LAUNCHED
1ST REQUEST FOR
INFORMATION (RFI)
FOR MPS
CHARACTERIZATION
STUDIES



COMPLETED
'LAB ON A CHIP'
SPECIAL EDITION
SERIES OF 9
MANUSCRIPTS

5

WORKSTREAMS

8

NEW ORGANOTYPIC
MANUSCRIPT
TEAMS



CONDUCTED
2ND LANDSCAPE
ASSESSMENT SURVEY
25 COMPANIES
RESPONDED





ORGANOTYPIC MANUSCRIPT TEAM

This team develops manuscripts that outline contemporary pharmaceutical industry considerations for developing, evaluating, and characterizing MPS models to support drug discovery and development. In 2020, the team completed a 9-manuscript series, which was published in the Royal Society of Chemistry's "Lab On A Chip" journal. In the coming year, the team anticipates supporting the development of 8 new manuscripts and publishing one manuscript that was initiated in 2020. These publications will expand on the first manuscript series by focusing on MPS models for additional organs and systems, including CNS/BBB, CNS/PNS, immune, ocular, and reproductive, and outlining considerations for using MPS to model diseases and develop new modalities such as oligos, gene, and cell therapies.

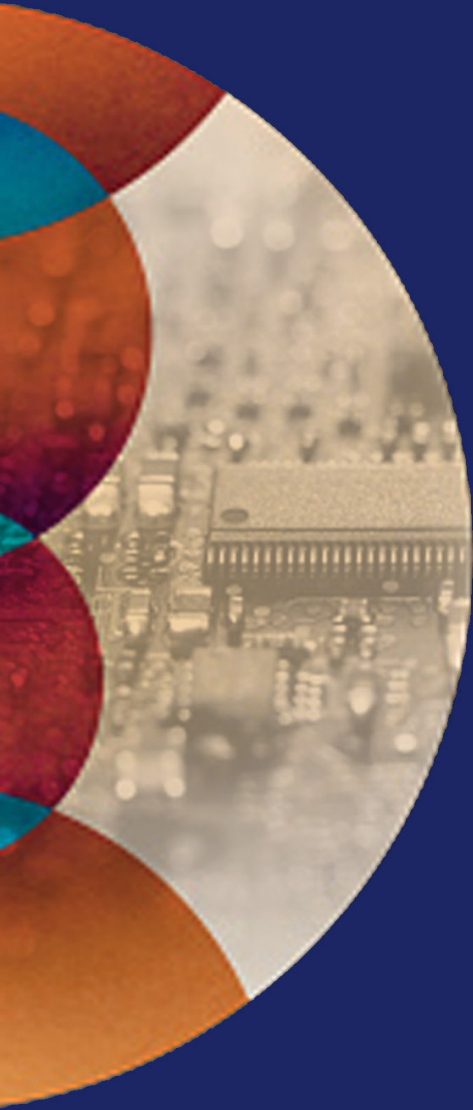
Key Accomplishments

- Completed a 9-manuscript series, which was published in the Royal Society of Chemistry's "Lab On A Chip"
- Formed 8 new manuscript 2.0 subteams and continued development of a CNS/BBB manuscript

Next Steps

- Develop 8 manuscripts for publication

LANDSCAPE ASSESSMENT SURVEY TEAM



For the second year in a row, the Survey team conducted a survey to assess the landscape of MPS technology use in the pharmaceutical industry and identify gaps between current MPS capabilities and the industry's needs. In 2020, the team facilitated several presentations on the results and key takeaways of the MPS Affiliate's inaugural Survey (Survey 1.0), including at the Feb. FDA workshop. The team also shared the survey analysis presentation with Affiliate member companies. In late 2020, the team distributed Survey 2.0 to IQ member companies. This revised survey was designed and refined based on Survey 1.0 results and FDA input and will allow IQ MPS to conduct a longitudinal analysis of changes in the field over the last year.

Key Accomplishments

- Presented Survey 1.0 Results at FDA F2F Meeting and PREDiCT: 3D Oncology & Tissue Models Summit

Next Steps

- Extract anonymized Survey 2.0 results in Q1 2021
- Complete Survey 2.0 analysis and summary in Q1 2021
- Perform longitudinal analysis from Survey 1.0 to 2.0 to evaluate the trajectory of MPS applications over time. IQ MPS will leverage these data to promote and advance partnerships and modify existing workstreams
- Prepare a publication using the longitudinal data to offer a commentary on the growth and application of MPS in drug discovery and development



COMMUNICATIONS & OUTREACH

The Communications Outreach team continued outreach to external organizations, and established dialogue with the European Organ-on-Chip Society (EUROoCs) and the National Centre for the Replacement, Refinement and Reduction of Animals in Research (NC3Rs). Collaboration with NC3Rs helped secure more responses to the Gut RFI.

Key Accomplishments

- Established collaborations with external organizations including EUROoCs and NC3Rs resulting in presentations to the IQ MPS Steering Committee
- Partnered with NC3Rs to secure more responses to the Affiliate's Gut RFI by advertising on NC3Rs' social media channels
- Maintained a website (<https://www.iqmps.org>) that drew 1,487 unique visitors in 2020 from around the world including the US, UK, Norway, Switzerland, France, Germany, Italy, Spain, India, China, and Japan

Next Steps

- Initiate contact with more external partners and grow membership
- Launch a virtual vendor fair proposal starting with an open call for presenters around a specified theme to learn about latest advances in MPS technology



JOINT CHARACTERIZATION OF MPS (RFI/RFP)

In 2020, the 'Joint Characterization (RFI/RFP)' Project team formulated and implemented a Request for Information (RFI) and Request for Proposal (RFP) process to facilitate cross-company collaboration and conduct prospective MPS studies. The team released an RFI for gastrointestinal (GI) MPS and received 11 responses from 8 organizations. Affiliate members are developing the prospective study design and considering RFI responses for advancement to the RFP stage. This team also initiated a collaboration with the Critical Path Institute's Predictive Safety Testing Consortium (PSTC) to develop and release a RFI for renal MPS.

Key Accomplishments

- Developed RFI/RFP process
- Released RFI for GI MPS and received 11 responses
- Initiated collaboration with PSTC for renal MPS RFI

Next Steps

- Determine details for RFP cost sharing and data ownership
- Develop a RFP for a specific GI MPS context of use and initiate at least one collaborative study
- Release renal MPS RFI in collaboration with PSTC

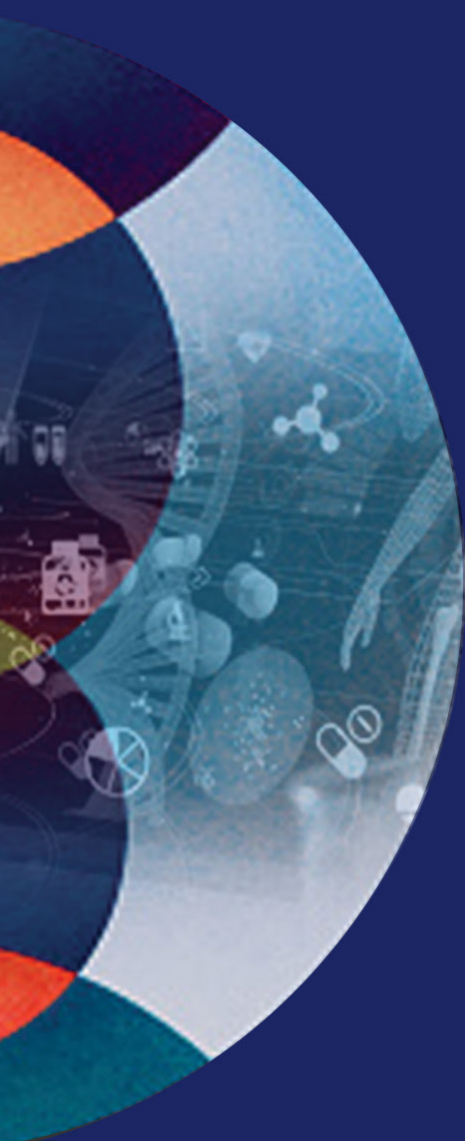
RETROSPECTIVE MPS DATA SHARING AND PROSPECTIVE MPS PROJECT ALIGNMENT

The Retrospective and Prospective MPS Data Sharing Pilot Project teams united forces in 2020 to successfully establish external discussion groups to explore opportunities for smaller company-specific collaborations involving retrospective data sharing or alignment in future MPS studies. With the support of Aim 1 and Aim 3 Pilot Project Teams, several discussion groups were formed, each focusing on a particular organ system or context of use of interest, and champions were identified to lead each group in exploring the opportunities for company collaborations.

Key Accomplishments

- Established framework for initiating collaboration discussion groups
- Provided considerations and guidance to the project champions and discussion group leaders on how to best navigate collaboration discussions
- Recruited project champions and initiated external discussion groups in Q4

REGULATORY ENGAGEMENT



In 2020, the Regulatory Engagement Team organized a successful workshop with FDA, which was attended by over 70 IQ MPS Affiliate subject matter experts and representatives from across the FDA's divisions. This event focused on overcoming hurdles to the adoption and acceptance of MPS technologies and on opportunities to overcome barriers to submitting MPS data in regulatory submissions. This team further continued its engagement with the FDA by organizing a joint webinar series, providing feedback on the FDA's MPS definition, and developing a position statement on animal cell-based MPS. IQ MPS representatives were also invited to present at the FDA Office of Clinical Pharmacology and IQ Consortium Scientific Exchange Meeting. In preparation for 2021, the team developed a strategy to establish similar relationships with other global regulatory agencies.

Key Accomplishments

- Held successful workshop with FDA
- Organized inaugural joint webinar with FDA and established plans to continue the series
- Provided feedback to FDA on their MPS definition
- Developed a position statement on animal cell-based MPS
- Presented at the FDA Office of Clinical Pharmacology and IQ Consortium Scientific Exchange Meeting
- Developed a strategy on engagement with global regulatory agencies beyond US

Next Steps

- Publish joint manuscript from 2020 F2F Workshop with FDA
- Publish a position statement on animal cell-based MPS in collaboration with FDA
- Continue to engage with FDA and build on our progress through joint webinar series and FDA workshop
- Establish similar relationships with other global regulatory agencies
- Identify path for ICH Guidance on MPS

WHAT OUR MEMBERS ARE SAYING ...



Szczepan Baran, MS VMD

Vice Chair (2021-2022)
Head of Emerging
Technologies, Novartis

"One of the most valuable benefits of IQ MPS Affiliate membership for us has been the opportunity to engage in constructive dialogue with regulatory authorities. Through workshops and webinars organized by the IQ MPS Affiliate, we've been able to gain actionable insight into FDA's perspectives on important topics, such as the burden of evidence needed to qualify MPS for specific contexts of use and how to build confidence in clinical relevance particularly with candidates in development. We look forward to the next event and to continue overcoming challenges to incorporating MPS data into regulatory filings."



Rhiannon N. Hardwick, PhD, DABT

Vice Chair Elect (2021-2022)
Senior Scientist,
Translational Safety Sciences,
Theravance Biopharma

"One of the reasons our company joined the IQ MPS Affiliate is the organization's ambition to accelerate MPS adoption and the opportunity to learn from the experiences of colleagues."

In the past year, the IQ MPS Affiliate reached a major milestone by issuing its first request for information to collaborate on evaluating GI MPS, which attracted a number of competitive responses. The IQ MPS Affiliate is now drafting a similar call for Kidney MPS in collaboration with the Critical Path Institute. We look forward to seeing both of these efforts enter the next phase and push towards proactive evaluation of MPS."



Diane Ramsden

Associate Scientific Director,
Takeda

"The IQ MPS Affiliate stands out from other organizations in the opportunities it creates to exchange insights with other experts in the field. As part of the Affiliate, I have actively engaged not only with a diverse group of scientists but also with developers and regulators through a number of highly interactive workshops. I've also had the opportunity to lead and participate in authoring IQ MPS-led organotypic, modality and context of use based manuscripts currently in process or published. Through these exchanges, the IQ MPS Affiliate is paving the way for the most promising applications for MPS by guiding the characterization data and features needed to successfully apply these tools during drug development and it has been amazing to be a part of it."

COMMENTS FROM MEMBERS ON THE VALUE OF PARTICIPATION

"The value of the IQ MPS Affiliate is that it provides opportunity to leverage the collective expertise of its members to help inform our company's efforts to implement MPS, as well as amplify our positions in conversations with developers and regulators. As an example, the organotypic manuscript series has enabled our company to exchange insights on the most promising applications for MPS and what is needed to realize their full potential with other experts in drug safety, ADME, and pharmacology. These manuscripts have also helped establish IQ MPS as a thought leader in this space by guiding the characterization data and features that are needed to successfully apply such tools in drug development."

"As our company looks to integrate MPS technology into our drug development processes, IQ MPS membership has been tremendously valuable to our company. We do not have a large, internal MPS group, and participating in discussions with other IQ MPS members and with regulators on how these systems are being used and viewed has given us tremendous insight and knowledge. Our participation in the IQ MPS has helped inform our efforts and connected us to the MPS community in ways that would not have been possible otherwise."

EXECUTIVE COMMITTEE

The Executive Committee provides leadership and guidance in the management of the business and affairs of the consortium between meetings of the Steering Committee, implements strategic plans recommended by the Steering Committee, and provides general counsel and tactical advice in support of IQ MPS's Working Groups.

PAST CHAIR 2020-2021

Terry Van Vleet
AbbVie

CHAIR 2020-2021

Jason Ekert
GlaxoSmithKline
Pharmaceutical R&D

VICE CHAIR 2020-2021

Szczepan Baran
Novartis

VICE CHAIR ELECT 2020-2021

Rhiannon Hardwick
Theravance
Biopharma

STEERING COMMITTEE

The Steering Committee provides strategic oversight of the initiative's portfolio and is the primary decision-making body for the IQ MPS Affiliate. The Steering Committee has representation from all member companies and acts on the recommendations of working groups and member companies to establish objectives, policies, and plans of action on a consensus basis.

ABBVIE

David Stresser
Terry Van Vleet

ELI LILLY AND COMPANY

Thomas Baker
Mike Mohutsky

NOVARTIS

Szczepan Baran
Patrick Devine

ALNYLAM PHARMACEUTICALS, INC.

Saket Agarwal
Chris MacLauchlin

GENENTECH, INC.

Aaron Fullerton
Will Proctor

PFIZER, INC.

Anna Kopec
Jennifer Liras

AMGEN

Xiaoting Wang

GLAXOSMITHKLINE PHARMACEUTICAL R&D

Julianna Deakyne
Jason Ekert

SANOFI

Piyush Bajaj
Karissa Adkins

ASTELLAS PHARMA, INC.

Sevim Yildiz Arslan

JANSSEN RESEARCH & DEVELOPMENT, LLC

Raymond Evers
Onyi Ofoma

TAKEDA PHARMACEUTICALS INTERNATIONAL, INC.

Diane Ramsden
Matthew Wagoner

ASTRAZENECA

Rhiannon David
Andrew Hall

MERCK & CO., INC.

Andreas Baudy
Jocelyn Yabut

THERAVANCE BIOPHARMA

Rhiannon Hardwick
Aaron Navratil

BIOGEN

Sandra Engle
Graham Marsh

MERCK HEALTHCARE KGaA

Philip Hewitt
Sakshi Garg

VERTEX PHARMACEUTICALS, INC.

Arek Raczynski
Sanjeev Kumar

BRISTOL MYERS SQUIBB

Silvi Chacko
Myrtle Davis

EISAI INC.

Tushar Kokate
Rongrong Jiang

MEMBER BENEFITS AND SECRETARIAT SUPPORT

IQ MPS is an Affiliate of the International Consortium for Innovation and Quality in Pharmaceutical Development (IQ) (<https://iqconsortium.org/>). The law firm of Faegre Drinker Biddle & Reath LLP serves as Legal Counsel and Secretariat to IQ.

Composed of attorneys, scientists, and project managers, Faegre Drinker Biddle & Reath's Consortium Management Team forms and supports life sciences industry collaborations that help companies throughout the world address topics of mutual interest. For the past 25 years, the team's work with these collaborations has yielded new scientific knowledge, sound, data-driven policies, and technological advances that improve the drug development process.

The Secretariat Supports the Affiliate By:

- Developing consensus positions on strategic initiatives and projects by facilitating member company decision-making processes within the IQ MPS
- Ensuring antitrust compliance by providing training, oversight, and ad hoc legal counsel
- Providing broad scientific, project management, legal, and administrative support
- Providing the Steering Committee with robust strategic, operational, and planning support, to include agendas, minutes, and presentations for meetings and teleconferences
- Supporting the exploration and scoping of data-sharing initiatives
- Reviewing manuscripts under development to ensure antitrust compliance
- Facilitating IQ MPS's external collaborations
- Managing internal and external communications
- Managing IQ MPS's website
- Providing venue and logistical support for in-person meetings

Member Companies Are Entitled To:

- Two seats on the Steering Committee
- Participate in all in-person meetings and teleconferences of the IQ MPS Affiliate
- Shape data-sharing and prospective collaboration projects
- Access to the IQ MPS Collaboration Site
- Nominate representatives to all workstreams and project teams (open to all company representatives)
- Contribute to ongoing organotypic manuscripts and propose new topics (open to all company representatives)



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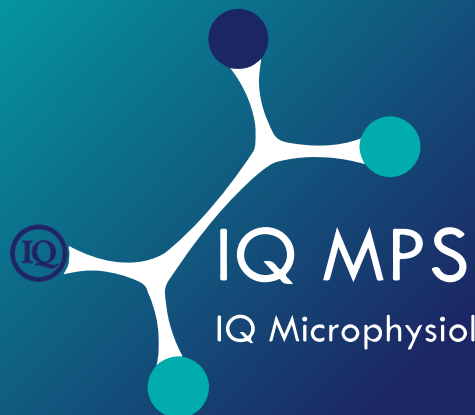


Catherine Graveline Mattia

Project Manager
Secretariat
catherine.graveline@faegredrinker.com

IQ MPS AFFILIATE MEMBERSHIP INQUIRIES

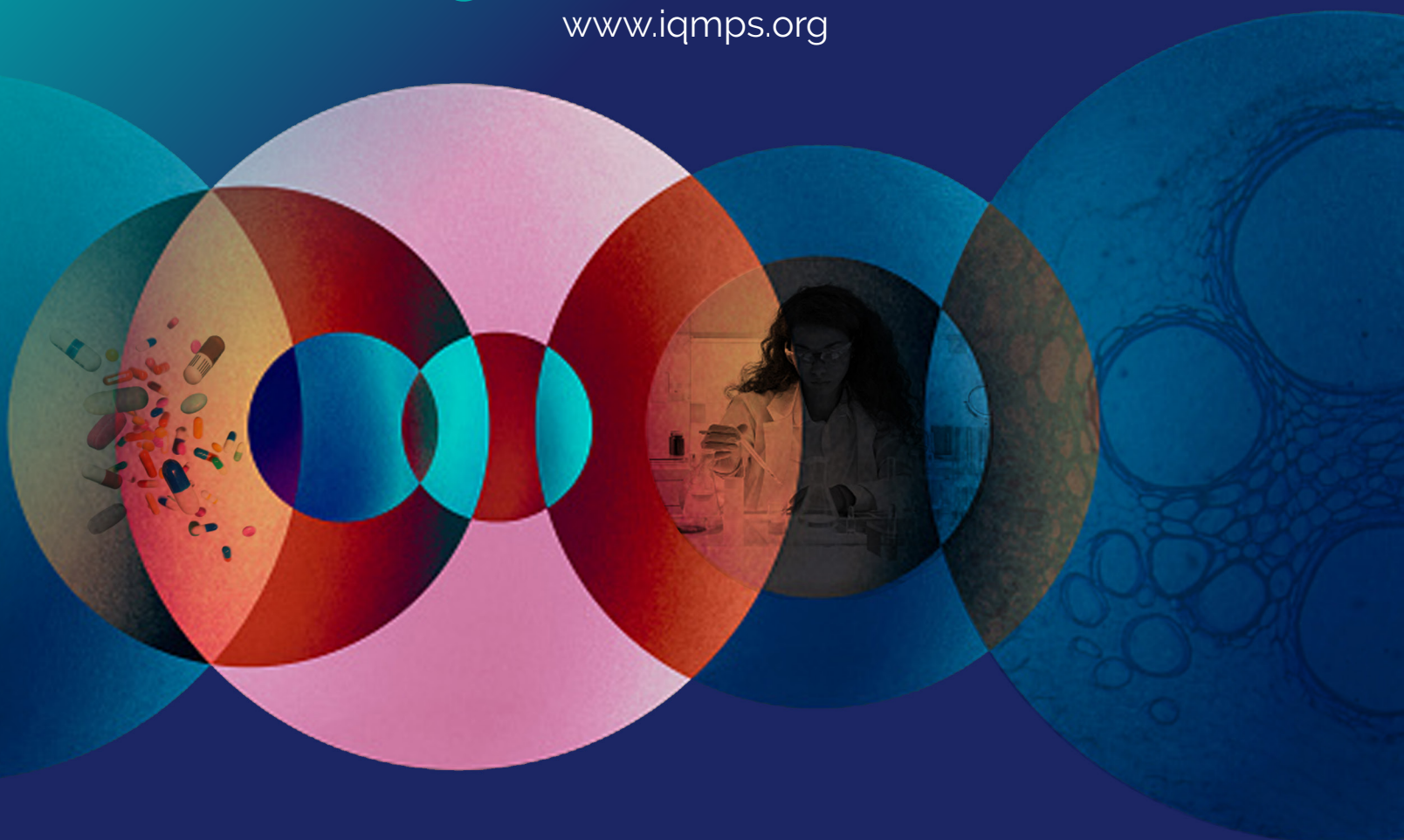
2021 Membership in the IQ MPS Affiliate is open to all members of the IQ Consortium. If you are not a member of the IQ Consortium but would like to learn more, we welcome the opportunity to provide an overview of membership options. For questions about the IQ MPS Affiliate and its priorities, progress and membership, please email **Catherine Graveline Mattia** at **catherine.graveline@faegredrinker.com**.



IQ MPS

IQ Microphysiological Systems Affiliate

www.iqmps.org



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