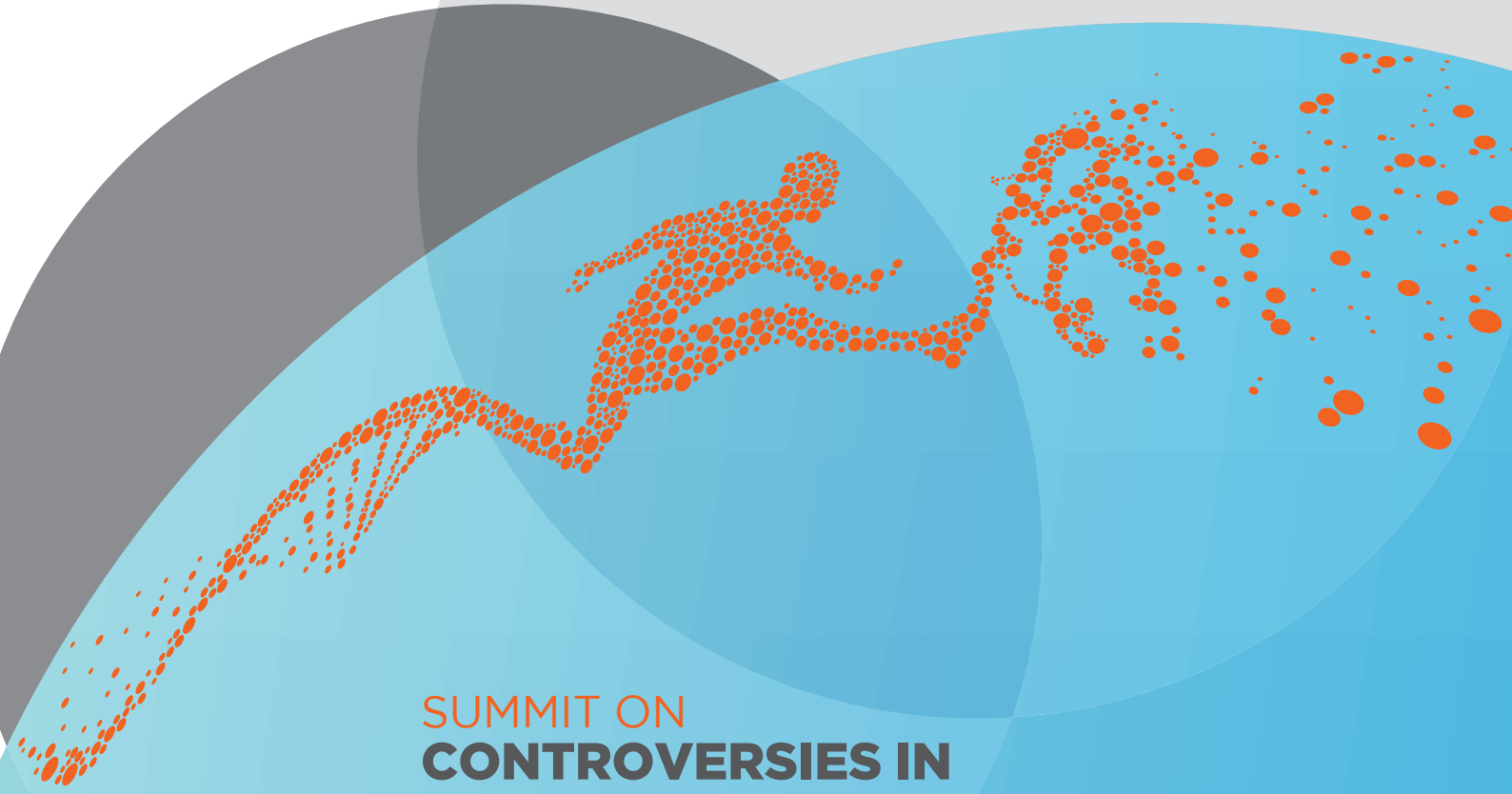
SCIENTIFIC PROGRAMME



SUMMIT ON
CONTROVERSIES IN
PRECISION MEDICINE

BERLIN, GERMANY
13-15 NOVEMBER 2017

WHERE DO WE STAND IN DRUG DEVELOPMENT?

ORGANISED BY
KENES GROUP

PrecisionMedicine.kenes.com

SUMMIT ON CONTROVERSIES IN PRECISION MEDICINE

ABOUT US

The growing controversies inside precision medicine need addressing head-on for the field to fulfil its promise.

Precision medicine aims to individualise therapeutic interventions, based on OMICS data such as genomics, proteomics, metabolomics etc', profiling together with histopathological insights to the type, stage, and the grade of the disease, as well as on the potential response of a particular patient to a particular treatment regimen. With next generation sequencing technologies, it is now possible to identify all germline variants of an individual at an affordable cost and thus paving the way for clinicians to provide healthcare from an individual perspective. However, the data that correlate specific disease type, response to specific treatment, and adverse effects as a result of the treatment, are slow to come. There are also critical voices warning us of getting lost in Big Data and advocating a stronger role of physiological models and phenotypic data in drug R&D. Another dimension of this debate is related to the way many companies manage their precision medicine processes and strategies for developing a more agile approach in the field.

The Summit on Controversies in Precision Medicine, under the leadership of Professor Dan Peer, Director of Tel Aviv University Cancer Biology Research Center and Translational Medicine, will explore in a realistic, non-biased manner global issues facing the community (clinicians, scientists, pharmaceutical industries and patients) that wants to build on the promise of Precision Medicine. By using plenary talks, scientific debates, and discussions, we will look at future ways to increase the productivity of biopharmaceutical and clinical innovation, with examples in oncology and rare diseases.

This year's summit is titled, "Where do we stand in drug development?" to create a focused environment for quality and open scientific debate on the value and direction of the industry. With an interactive architecture, the event is designed to provide the participants with exciting opportunities to ignite solutions, network and innovate.

Become part of 150+ senior level attendees from leading industry companies, academia and government institutions who have put innovation in their pipeline.

If you are eager to explore strategies for addressing challenges and igniting solutions in Precision Medicine, join us this November in Berlin!

OUR GOALS

- To provide participants with tools to ignite solutions in their organizations
- To create a focused environment for quality debate on the value and direction of precision medicine
- To generate deep understanding of biology vs clinical impact
- To present the two sides of the same coin and spark the conversation between industry, academia and policy makers.
- To provide perfect networking opportunities for industry stakeholders, policy makers and academic counterparts.

WHY ATTEND?

- Develop the tools you need to lead precision medicine in your organization
- Get access to focused and provocative discussion on the scientific, technological and practical challenges in precision medicine
- Be part of engaging debates, panels and roundtable discussions which will help you maximize your time and experience at the Summit
- Delve into the real challenges and opportunities in the industry that will enable change
- Get access to quality networking with colleagues facing the same challenges and meet thought leaders who will share invaluable insights on precision medicine

WHO WILL ATTEND?

Be part of 150+ senior level attendees from leading industry companies, academia and government institutions who have put innovation in their pipeline.

Biopharma & Tech Executives

CROs & R&D Leaders

Solution Providers

Academic Dept. Heads

Research Teams

Gov. Innovators & Investors

SCIENTIFIC ADVISORY BOARD

Dan Peer
Director, Tel Aviv University Cancer Biology Research Center, Tel Aviv, Israel

Christian Elze
Senior Partner Catenion, London, UK

Gianni Gromo
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Michael Heuser
Heisenberg Chair for Molecular Therapies in Hematology, Hannover Medical School, Hannover, Germany

Yelena Yesha
Professor, Department of Computer Science and Electrical Engineering, University of Maryland, Baltimore County, USA

REGISTRATION

	Registration before 24 October, 2017	**Registration from 25 October, 2017**
Commercial Participants	€1590 + VAT	€1900 + VAT
Academic Participants	€670 + VAT	€800 + VAT
Clinician Participants (HCPs)	€670 + VAT	€800 + VAT
Packages including Hotel	Minimum 2 nights 13-15 November €140 per night inc breakfast, VAT, city tax	n/a
Discounts on registration fees (not inc hotel)	20% reduction for groups of over 3 participants	20% reduction for groups of over 3 participants

German VAT = 19%

To register, please visit the website: PrecisionMedicine.kenes.com

MONDAY, 13 NOVEMBER 2017

THE DEBATE: CONTROVERSIES IN PRECISION MEDICINE 16:00-18:00

We will introduce why and how we are at point of controversies today. The opening debate will explore whether a genetic-driven approach just needs more time to produce results or whether we need to first invest more in a regulatory filtering of genetic variations.

WELCOME FROM SUMMIT CHAIRPERSON

Dan Peer
Director, Tel Aviv University Cancer Biology Research Center, Tel Aviv, Israel

OPENING ADDRESS:

Martin Lohse
Chair of the Board of Directors and Scientific Director of the Max Delbrück Center for Molecular Medicine in the Helmholtz Association Member of the Board of Directors of the Berlin Institute of Health (BIH), Berlin, Germany

Richard FitzGerald
Director, Clinical Research Unit, RLUH, Liverpool, UK

Same Medicine, Different Results - the Impact of Personalised Medicine
The talk will highlight the origins and rationale behind personalised medicine, its current reach and the potential for personalised medicine to transform the way care is delivered to patients over the next two decades .

Denis Noble
Emeritus Professor of Cardiovascular Physiology, Oxford, UK

Regulatory Filtering of Genetic Variation: Why GWAS Outcomes so far are Disappointing
The influence of variations in DNA sequences on the phenotype is strongly filtered by regulatory networks at higher levels (cells, tissues, organs, systems). This is the main reason why GWAS research has produced relatively disappointing outcomes in terms of correlations with health and disease. It is now far from certain that extending such studies to much larger cohorts will solve the problem. The solution may be to switch to understanding better the filtering by regulatory networks.

PANEL DISCUSSION

PLENARY LECTURE: BUSINESS PERSPECTIVE

18:00-19:00

How to bring precision medicine to fields outside of oncology, mendelian rare diseases and infection diseases. We will review what has worked or not worked to date and therefore factors we need to be considering in moving forward. We will hone-in on how to approach building more viable businesses in precision medicine.

Christian Elze
Senior Partner Catenion,
London, UK

New Business Models for New Drugs
The economics of the innovation model built on private risk capital and biotech firms for translation are unsustainable.
To ensure broad patient access to innovation, we need new forms of financing research, translating research findings into therapies and delivering these therapies to patients.
The talk will cover rare diseases, antibiotics and gene & cell-based therapies.

Catenion

Robert Wasserman
Chief Medical Officer
at Northern Biologics,
Toronto, Canada

Biomarker-driven Oncology Drug Development: From Pharmaceutical Giants to a Start-up Biotech.
In his presentation, Robert Wasserman will discuss his experiences developing Oncology drugs in regard to Precision Medicine approaches from his time at Novartis and Roche to his current employment at Northern Biologics. Specifically, he will compare and contrast both the scientific approaches and the dedicated resources available to execute on a personalized drug development pathway.

NORTHERN BIOLOGICS

NETWORKING RECEPTION

19:00

TUESDAY, 14 NOVEMBER 2017

PRECISION MEDICINE – THE PHARMACEUTICAL STANDPOINT 08:30-10:15

Pre-Disease Intervention: Bringing Precision Medicine from the Back End to the Front End of Diseases. We will focus on how industry perceives the current challenges in precision medicine and their outlook based on specific case studies.

Thomas Bock
Chairman and Chief Executive Officer HeritX, Santa Monica, USA

Preventing Cancer – Turning a Challenge into Unprecedented Opportunity
Unlike other cancers, we already know the first step in the development of BRCA cancers (the inherited BRCA mutation), can identify BRCA mutation carriers through genetic testing, and focus our unprecedented biomedical tools and technologies to medically intervene before cancer starts. In BRCA, prevention is possible. Moreover, similarities between BRCA pre-cancer and the development of other cancers will make advances in BRCA applicable to other cancers: BRCA is a gateway to cancer prevention.

Matthias Ocker
Head of Biomarker Strategy, Bayer AG, Berlin, Germany

Biomarkers in Oncology Drug Development
Current oncology drug development aims to bring effective and safe drugs to a molecular defined subset of patients. Biomarker research and companion diagnostics development thus increase the overall success rate of drug development and parallel the classical drug development value chain. The maturation of non-conventional drugs and drug target is therefore dependent on this close co-development. As the current diagnostic environment is rapidly changing by taking up also panel approaches to identify rare patient populations, industry, academia as well as regulators and public institutions need to better align on novel strategies to successfully develop these new drugs.

Ludwig Heesen
Project Manager Clinical Studies, BioNTech, Mainz, Germany

Novel RNA-Based Cancer Immunotherapies
Immunotherapy has evolved as a promising alternative to conventional treatments against cancer. Vaccination with RNAs encoding for tumor-specific antigens or neo-epitopes has been shown to be capable of efficiently inducing antigen-specific T-cell responses and anti-tumor immunity. We have developed pioneering RNA-lipoplex (RNA(LIP)) immunotherapeutics for intravenous application which are currently being tested in clinical trials with promising preliminary results.

ROUND TABLE DISCUSSION

COFFEE BREAK, NETWORKING, EXHIBITION 10:15-10:45

PRECISION MEDICINE IN THE CLINIC – CLINICAL STANDPOINT 10:45-12:35

Case Studies & Panel Discussion on the clinical benefit
We will seek to understand when a precision medicine approach will be beneficial and when it is unlikely to be so. If we can increase our understanding, then we can better focus on increasing the efficiency of precision medicine based drugs.

Ido Wolf
Head, Oncology Division, Tel Aviv Medical Center, Tel Aviv, Israel

Molecular Tests in the Clinic: Where Do We Stand in 2017?
Precision medicine is being perceived by patients and physicians alike as a new standard of care in oncology. However, the definition of precision medicine is vague and the evidence regarding its benefit in the clinic are scarce and often controversial. In his talk, Prof. Wolf will mostly refer to genomic testing and use evidence-based approach to discuss if and how genomic tests should be routinely used in the clinic. Another topic to be discussed is the use of data generated routinely in the clinic, as an accelerator of basic science research.

Michael Heuser
Heisenberg Chair for Molecular Therapies in Hematology, Hannover Medical School, Hannover, Germany

Precision Medicine in the Academic Setting
How precise should precision medicine be? One patient has several targets and tumor cells may rapidly evade the selective pressure of a specific inhibitor. Michael Heuser will discuss what can be achieved with a molecularly targeted approach in a concerted action of the national and global leukemia community.

Pia Raanani
Head of The Hematology Devision in Rabin Medical Center, Tel Aviv, Israel

Personalized Medicine in Chronic Myeloid Leukemia (CML) in 2018 – Does It Exist? A Clinician's Perspective
Unlike its (controversial) place in the treatment of solid tumors, personalized medicine in hemato-oncology is still in its infancy.
Bearing this in mind, we ask ourselves whether there is a role for precision medicine in CML at all and if yes, what are its implications - Scientific? Clinical? Both?
The lecture will concentrate mainly on the clinical aspects of personalized medicine in CML. Cutting edge objectives in CML in 2018 and their applications for a personalized approach will be discussed including: the various options for first line treatment, adherence to treatment, treatment free remission (cure?) and treatment discontinuation, age adjusted treatment according to: therapeutic goals, comorbidities and adverse events, the issue of pregnancy in young CML female patients, and finally, the role of bone marrow transplantation, once the leading approach in CML, in the era of targeted therapies and precision medicine.

Ulrich Keilholz
Acting Director, Charité Comprehensive Cancer Center, Berlin, Germany

Precision oncology 2018: bridging targeted treatments and immunotherapy

Precision cancer medicine is exciting and rapidly developing, but also had to take several steps in terms of complexity. And there are several levels still ahead. A major current question is the integration of targeted and immunotherapeutic approaches into clinical precision medicine programs. This interaction between signal pathway drugs and immunotherapy offers clinical advances as well as improves our understanding of the complex tumor host interactions, which will be detailed in the presentation.

ROUND TABLE DISCUSSION

LUNCH, NETWORKING & EXHIBITION 12:35-13:35

TUESDAY, 14 NOVEMBER 2017

PATIENT STRATIFICATION VIA GENOMICS/EPI-GENOMICS IN CANCER 13:35-15:25

Through the power of genomics and epi-genomics, we are starting to see some success in patient stratification for treating cancer. However, we currently lack the necessary tools to fully process and analyse the data in many cases, such as when multiple patients' phenotype data does not correspond to shared genome level mutations. How can we improve our data and what next steps do we need in clinical informatics?

Erez Levanon
Associate Professor at The Mina and Everard Faculty of Life Sciences, Bar-Ilan University Ramat Gan, Israel

RNA Editing - Common, Hidden Cancer Mutations
RNA editing alters the mRNA sequence from its genomic blueprint in a dynamic and flexible way. We show that A-to-I editing and the enzymes mediating this modification are significantly altered, usually elevated, in most cancer types. Increased editing activity is found to be associated with patient survival. As is the case with somatic mutations in DNA, most of these newly introduced RNA mutations are likely passengers, but a few may serve as drivers that may be novel candidates for therapeutic and diagnostic purposes.

Genomics in Precision Medicine. Clinical Informatics

Xiangdong Wang
Director Shanghai Institute Clinical Bionformatics, Fudan University Zhongshan Hospital, Shanghai, China

Clinical Bioinformatics in Lung Diseases
The importance and application of clinical bioinformatics are growing with the development of omics-technologies. We have organized global opinion leaders to write a series of Translational Bioinformatics to address the concept and content of clinical bioinformatics. We have applied clinical bioinformatics as a tool to analyze genomic and proteomic profiles, metabolic and signaling pathways, biomarker discovery and development, human tissue bank establishment, as well as precision medicine in lung cancer and chronic obstructive pulmonary diseases. Based on clinical bioinformatics, we are investigating and integrating networks of genomics, proteomics, metabolomics, lipidomics, and microbiomics in chronic lung diseases to discover new disease-specific biomarkers and precision therapies.

Manfred Dietel
Full Professor and Director of the Institute of Pathology, University Hospital Charité, Berlin, Germany

Morpho-Molecular Pathology as Prerequisite for Precision Medicine in the Clinics
Research orientated molecular and immunological work and clinically relevant applications for the individual patient are two approaches. The representatives sometimes think in different ways. In particular, the (tumor) material used for the analyses must be carefully selected and controlled by morphology prior to serve as basis for research or treatment decisions. Thus, it is of crucial importance that both sides of the coin understand each other.

Deepak Asudani
Associate Clinical Professor of Medicine, Vice Chief, Clinical Operations, Division of Hospital Medicine, University of California San Diego, USA

Clinical and Genomic Context in Druggable Targets and Refining the Evidence Base
The patient stratification based on genomics/epigenomic profile in drug development promises tremendous impact in the way we define numbers needed to treat and numbers needed to harm. In a lot of ways, inclusion of stratification based on genomics and epigenomic data is a 'reset point' for clinical trials and provides a renewed perspective in the way we conduct studies. This is especially more important in drug development for cancers given a myriad array of genetic expression in development and evolution of diverse cancers. Besides diagnostic, predictive and prognostic value the epigenomic data has compelling value in patient stratification.

ROUND TABLE DISCUSSION

COFFEE BREAK, NETWORKING, EXHIBITION 15:25-15:55

DEVELOPING NEW DELIVERY VEHICLES FOR PRECISION MEDICINE 15:55–17:40

Much of the potential of precision medicine rests on drug delivery strategies. What is achievable with current drug carriers and where do we need to focus in our current pipeline? How can precision medicine benefit from nanomedicine? And if the future of precision medicine lies in altering nucleic acid, then where do we need to focus our research to make it a reality?

Heinrich Haas
Vice President RNA Formulation & Drug Delivery at BioNTech RNA Pharmaceuticals, Mainz, Germany

Engineering Nanomedicines for RNA Delivery in Tumor Immunotherapy
In this session we give insight into the targeting coherencies within intravenously injectable messenger RNA (mRNA) lipoplex nanoparticle formulations, applicable for tumor therapy and further therapeutic approaches. Accurate control of the nanoparticulate organization at the molecular scale enabled design of vehicles for efficient targeting of different organs with high selectivity. Methods for semi-automated manufacturing of personalized vaccines for tumor immunotherapy as ready-to-use products could be developed. Several clinical trials have been initiated or are underway.

James Taylor
Co-founder, CEO at Precision NanoSystems, Inc., Vancouver, Canada

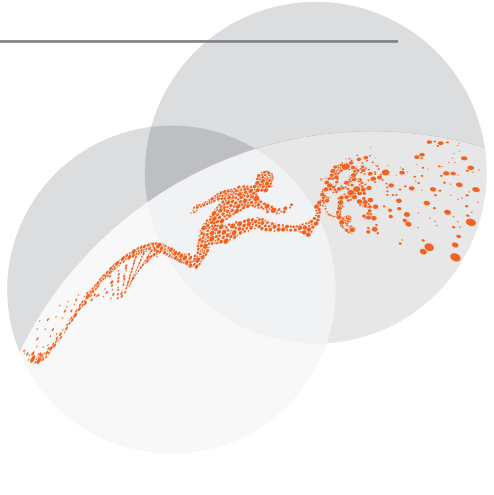
Rapid Development and Scalable Manufacture of Precision Nanomedicines
The past decade has seen the rapid development of new powerful therapeutic modalities, including advances in small molecule, protein, and nucleic acid based drugs with the potential to treat disease at the molecular level. Nanoparticle delivery is a key enabling technology to transfer these new therapeutic advances into clinical realities. This talk will present the use of continuos-flow microfluidic manufacturing for the rapid and scalable development of nanomedicines.

PRECISION NANOSYSTEMS
Create Transformative Medicines™

Twan Lammers
Head of department of Nanomedicine and Theranostics, Institute for Experimental Molecular Imaging, RWTH Aachen University Clinic, Aachen, Germany

Towards Precision Nanomedicine
Hundreds of tumor-targeted drug delivery systems are developed every day. However, hardly any of these so-called nanomedicine formulations have been approved for clinical use. This is likely largely due to the lack of (imaging) biomarkers to pre-select patients for such targeted nanomedicine treatments. In this lecture, Twan Lammers will discuss strategies to improve the performance and clinical translation of tumor-targeted nanomedicine, and explain how imaging can be used to individualise and improve nanomedicine treatments.

ROUND TABLE DISCUSSION



THE FUTURE OF PRECISION MEDICINE FROM A REGULATORY STANDPOINT

17:40-18:40

Regulatory agencies will present their view on the emerging field of precision medicine and what they are seeing as the needed criteria to ensure a fair and efficient approval process.

Khaled Bouri
Science Advisor Office of Regulatory Science and Innovation, Food and Drug Administration, Silver Spring, USA

Regulatory Considerations of the Use of Next Generation Sequencing and Biomarkers to Support the Implementation of Precision Medicine
The discussion will focus on FDA efforts to advance regulatory science to support the review and evaluation of Next Generation Technologies, and the use of biomarkers in clinical drug development. The discussion will also address FDA efforts to collaborate with other federal agencies, academic institutions, and other stakeholders to address scientific and regulatory challenges related to precision medicine.

Christopher Rowe
Innovation Lead, Precision Medicine, Innovate UK, Swindon, UK

The Role of Innovate UK in the Development of Precision Medicine
Innovate UK is the UK's innovation agency. We have a strong business focus and a track record of driving growth by working with companies to de-risk, enable and support innovation – putting them in a stronger position to attract investment and facilitating access to investors, collaborators, customers and export markets. We have an interest in the area precision medicine as part of the Health & Life Science sector portfolio. We perform a range of activities to help companies working in the precision medicine area, including a range of funding opportunities. We also work with a wide range of stakeholders that can help companies develop their value proposition and technology and help companies to work with these organisations. This presentation will review the work of the Precision Medicine team and how it helps companies develop precision medicine technologies in the UK.

WEDNESDAY, 15 NOVEMBER 2017

DISEASE SIGNATURE AND SMALL VS. BIG DATA IN PRECISION MEDICINE 08:30-10:15

This session will review the challenges of operating without an aligned definition of disease signature. We will consider the amount of data we are processing, both small and big data, as well as whether we should be taking advantage of clinical trials to collect as much real-time physical and environmental data.

Mira Marcus-Kalish
Director, International Research Affairs, Tel-Aviv University Ramat Aviv, Tel Aviv, Israel

Big vs. Small Data Analysis Targeting "Disease Signatures" Towards Precision Healthcare
A 3C- Categorization, Classification & Clustering- strategy, aims to provide a comprehensive insight through a stepwise process, incorporating medical expert knowledge in a structured way into the analysis process of the disease manifestations and potential biomarkers, towards reliable precise medicine.
Two case studies utilizing the 3C strategy, will be presented in this session. A Parkinson's disease cohort of diagnosed patients and their family members yielding new genotypes associations and the Alzheimer's disease Neuroimaging Initiative (ADNI) cohort going beyond the discovery of association to new sub-type identification.

Yelena Yesha
Professor, Department of Computer Science and Electrical Engineering, University of Maryland, Baltimore County, USA

A Personalized Approach to Medicine: Utilizing Accelerated Real-Time Analytics for Clinical Data
Large scale healthcare data analysis is a budding area of research currently nurturing a bunch of cross-disciplinary enterprises poised to change the daily course of everybody's life. A cornucopia of biomedical applications born in the wake of the postgenomic revolution recently became a driver for long-awaited implementation of the holistic vision of human health and wellbeing. Data-enabled science ("BigData") borrows its insights from the adjacent fertile fields of genomics, proteomics and metagenomics, transcriptomics and metabolomics, systems biology and bioinformatics. Now it is about time to see postgenomic ideas come to fruition through an acceleration of drug discovery and its time to market, providing means for staving off diseases by watchful maintenance of health and, last but not least, charting the path for healthy aging.

Ancha Baranova
Associate Professor, School of Systems Biology, George Mason University, Fairfax, USA

Step Away of the Edge of an Illness: Longitudinal Monitoring and Abatement of Stress
Slow Progress of Systemic Inflammation, Insulin Resistance and Metabolic Deficiency serves as the most reliable sign of ageing. These processes directly contribute to pathophysiological course of many chronic diseases. Their hallmarks serve as surrogate biomarkers which could be traced in advance of symptomatic stage. In frame of disease prevention paradigm, when these biomarkers trend away from their optimums, means of the physiological control should be used in longitudinal management of health and as an aid to healthy ageing.

ROUND TABLE DISCUSSION

COFFEE BREAK, NETWORKING, EXHIBITION 10:15-10:45

WEDNESDAY, 15 NOVEMBER 2017

HEALTH INFORMATICS 10:45-12:30

How do we analyse complex diseases? Are computer-based solutions such as AI and machine learning the best solution direction? What is their potential and limitations? And what future steps do we need in R&D to actualise such solutions?

Harsha Rajasimha
Global Head, Life Sciences R&D, NTT DATA Inc.,Washington, USA

Patient Centricity in The Era of Data-Driven Value-Based Precision Medicine
The growing number of FDA approved targeted therapies on one-side and an increasing number of pay-for-performance deals between big pharma and payers on the other, indicates that the life science industry is heading towards a data-driven, value-based, precision medicine. In order to adapt to this new paradigm, life science companies need access to RWD (Real World Data) and the ability to draw insights for clinical R&D, value-based pricing negotiations and better reimbursements. Insights drawn from RWD are expected to enable biopharma companies to better engage with the patients directly eliminating the traditional bottlenecks. Harsha Rajasimha will present his point of view on how Biopharmaceutical companies are adapt to these and other major trends to sail through the storm and present potential solutions to address use cases in R&D, commercial and value-based reimbursements.

Sahar Gelfman
Research Associate Institute for Genomic Medicine, Columbia University Medical Center

Identifying Non-Coding Variants that Cause Rare Disorders: Opening a Window to the Rest of the Genome in Variant Detection Analyses
Identifying the underlying causes of disease requires accurate interpretation of genetic variants. Current methods successfully capture the effect of coding variants, but ineffectively capture pathogenic non-coding variants in genic regions. For this reason, synonymous and intronic variants are mostly overlooked when searching for disease risk, although they may infer severe damage to the protein by damaging the final transcript. Here we present the Transcript-inferred Pathogenicity (TraP) score, which uses sequence context alterations to reliably identify non-coding variation that causes disease. TraP accurately distinguishes known pathogenic and benign variants in synonymous (AUC = 0.88) and intronic (AUC = 0.83) public datasets, dismissing benign variants with exceptionally high specificity. TraP's strong advantage in finally allowing for the inclusion of these sites in gene discovery and diagnostic sequencing efforts is already being utilized at institutes around the world to perform genetic diagnosis and identify risk factors in large disease cohorts such as ALS, epilepsy, Parkinson's disease and schizophrenia.

Edit Buzas
Professor and Chair at Semmelweis University, Department of Genetics, Cell- and Immunobiology, Budapest, Hungary

Extracellular Vesicles: Next Generation Tools in Precision Medicine?
Extracellular vesicles such as exosomes, microvesicles and apoptotic bodies are recently recognized subcellular structures of cell-cell communication. Their potential exploitation as novel biomarkers and/or therapeutic tools place them into the focus of intense current research. The talk will summarize the strengths and limitations of extracellular vesicles with respect to their potential use in precision medicine.

ROUND TABLE DISCUSSION

LUNCH, NETWORKING & EXHIBITION 12:30-13:30

THE POWER OF PRECISION MEDICINE IN RARE DISEASE 13:30-14:30

Panel discussion: The Role of Foundations in Precision Medicine

The field of rare diseases is expanding to the point of more than 10,000 sub-diseases. There is a lack of single drugs suitable for enough of these sub-diseases, which has made the drug discovery process less financially attractive and riskier for pharma. The challenge for precision medicine is to develop a global drug solution where there is a common denominator. Where are we at and how can the market move forward?

Peter Kapitein
President and Patient Advocate Inspire2Live, Amsterdam, Netherlands

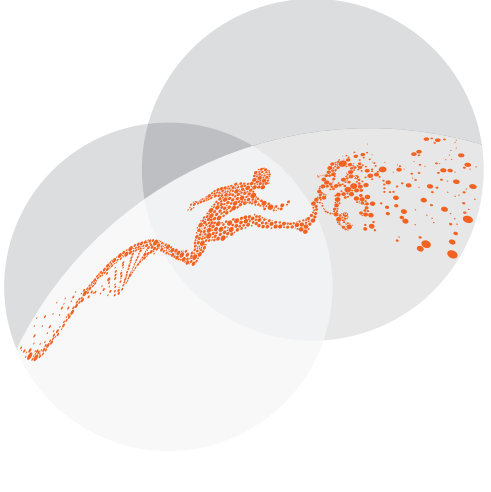
The Importance of Precision Medicines – Hurdles and Solutions
Precision medicine is already a success and nevertheless it might fail. We can learn from the past and should implement precision medicine not as just another way of treating patients but as another and new approach. In this new approach patients play an important role and are very motivated to do so. The biggest hurdle to take however is the medical industrial complex. Will this complex be prepared to innovate or will they simple stick to their old way of doing things?

Janet Allen
Director of Strategic Innovation at Cystic Fibrosis Trust, London, UK

Precision Medicine for Cystic Fibrosis: Each Genotype Defines New Therapeutic Opportunities
In 2012, a new treatment (ivacaftor) was launched that has a transformational effect on people carrying a rare mutation, G551D. This represents only about 400 of the 10,800 people with cystic fibrosis in the UK.
This discovery has improved enormously our understanding of the condition and the mutations that change the function of the protein. This has allowed the drug, ivacaftor, to be approved for use in a significant number of other mutations beyond G551D.
Cystic fibrosis is therefore a condition where the nature of the genotype defines the therapeutic option for individuals and new drugs have changed the understanding of the condition.

Lior Dor
Founder of Ran's smile – the Israeli STXBP1 foundation, Rehovot, Israel

Living with a Child with a Rare Disorder and Advancing Research for a Cure
STXBP1 disorders are very rare leading to neonatal epilepsy, global delay, movement disorders and etc. After getting our son's diagnosis we found that there were only 7 reported cases of patients with the same specific mutation. We realized that the medical community has very little experience with this disorder and that research is focusing mainly on basic science. We then decided that it is "up to us" to advance research for finding a cure by contacting researchers and generating a foundation dedicated for this cause.



WEDNESDAY, 15 NOVEMBER 2017

NEW THERAPEUTIC MODALITIES FOR RARE DISEASE 14:30-16:15

Is there potential for a new protein or messenger RNA that will fit all mutations and generate a global solution for a disease? Case studies on this and other novel approaches for treating rare diseases

Eran Blaugrund
VP R&D, ART BioScience Ltd, Rehovot, Israel

Modified mRNA Therapeutics for Rare Diseases
The field of rare diseases is turning out to be a lucrative area if breakthrough medications can be provided to patients. In addition, regulatory agencies are trying to support and facilitate the development process for these disorders. This has brought about a large number of novel strategies for Duchenne muscular dystrophy (DMD) drug development but it remains to be seen whether these innovations can really make a difference in patients` lives'. Eran Blaugrund will provide a critical view on some of these novel methodologies and focus on one of them, injection of dystrophin mRNA.

ART BIOSCIENCE
BRIDGING THE THERAPETIC GAP

Stuart Hughes
Director and Head of Pharmacology at Vertex Pharmaceuticals, London, UK

Leveraging Known Causal Human Biology for Discovering New Medicines for Orphan Diseases
In many cases the underlying cause of orphan and rare diseases is known, often being genetic nature. However, that the trajectory of disease causing events and a validated therapeutic intervention point are also known does not always follow. I will discuss the key requirements and approaches for addressing diseases where the essential causative events are known as well outline how this should differ for diseases where this is not the case.

Thomas Wilckens
CEO at InnVentis Ltd., Kadima, Israel

How Rare Disease, Omics and AI can Help to Identify New Drug Targets or Nutraceuticals for Chronic Inflammatory Conditions
The contemporary drug discovery and development process seem to not sustainable not only for the cost explosion, but also regarding the efficacy of translating early preclinical data, often generated in animal models into a successful clinical development path. In fact both, target as well as biomarker discovery success rates are pathetically low. This is not only attributable to the fact that up to 85% of preclinical research is not reproducible, but also the very poor correlation of complex dynamics regulation of biological processes from animals (mainly rodents) with the human system. New paradigms using longitudinal data collection with multi-omics approaches to decipher the complex pathways regulating homeostasis and the development of pathologies gain more and more traction, in particular regarding multi-factorial polygenetic inflammatory/autoimmune conditions we today call for example “rheumatoid arthritis”. In these polygenetic conditions the contribution of genetic background to the development of disease as well as delineation of new routes of intervention remains challenging. However, there are also monogenetic conditions termed autoinflammatory conditions like Familial Mediterrannean Fever, Blau Syndrome, Still Syndrome and others, that in comparison with other chronic inflammatory conditions may allow insight into the deviation of pathologies from the maintenance of homeostasis, in particular with regard of the contribution of the inflammasome versus the immune system to a given pathology.

ROUND TABLE DISCUSSION

COFFEE BREAK, NETWORKING, EXHIBITION 16:15-16:45

CLOSING - SUMMARY & DISCUSSION 16:45-17:15

Panel Discussion on the next steps for precision medicine. Summary of where are the challenges and what are the potential solutions.

SUPPORT & PARTICIPATE

You can choose from the packages below or from the list of opportunities below. Your level of support will be determined by your total contribution (including support opportunities and exhibition space).

Level of Support	PLATINUM	GOLD	SILVER
Total Contribution (+VAT*)	**€ 20,000 +**	**€15,000**	**€8,000**
BENEFITS:			
Speaker Presentation (30 min)	✔	✔	
Research / Product Theatre (15 min)	✔		✔
Roundtable Host (30 min)		✔	
Advertisement (in programme book)	✔	✔	
One Exhibition Counter	✔		✔
Full Registration(s)	2	1	
Delegate List (name and country)	✔	✔	✔

Support Opportunities:	Price (+VAT*)
Speaker Presentation (30 min)	€10,000
Research / Product Theatre (15 min)	€5,000
Roundtable HOST (30 min)	€8,000
Mobile App	€20,000
Delegate Bags	€5,000 + provided in kind
Lanyards	€6,000 + provided in kind
Welcome/Networking Reception	€12,000
Branded Seats in Exhibition (50)	€8,000
Branded seat covers (in the session hall)	price upon request
Advertisement (inside page of programme book)	€2,000
Meeting Rooms/ Hospitality Suites	price upon request

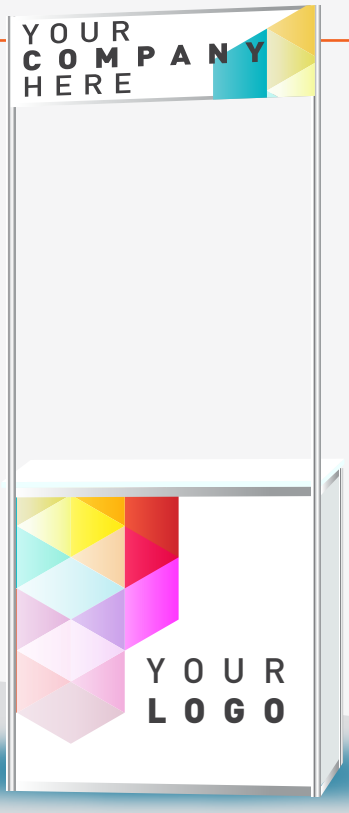
INDUSTRY HUB €5,000

WE ARE OFFERING THE NEW HIGH COUNTER EXHIBITION BOOTHS

- High counter with your branding and high chair
- Two Exhibitor Badges
- 100 word company/product profile in programme
- Cleaning of public areas and gangways
- Invitation to the Welcome Reception for registered exhibitors

Other furniture or electrical usage will not be included. All these services and other will be available to order in the Exhibitors' Technical Manual.

Larger exhibition spaces also available, upon request.



We would be pleased to discuss with you and customize a package to your needs.

CONTACT US to schedule a call, for more information and for bookings:

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