BOOK BY 30TH OCTOBER AND SAVE £400 | BOOK BY 30TH NOVEMBER AND SAVE £200 | BOOK BY 18TH DECEMBER AND SAVE £100

"Overall very good organisation/speakers" - ***GSK***

SMi
LINKING BUSINESS *with* INFORMATION

SMi presents the 7th annual conference on...

RNA Therapeutics

Sustainable production of targeted delivery and mRNA

Holiday Inn, Kensington Forum, London, UK

15 - 16 FEB 2016

Why you should attend:

- Learn new **Pre-clinical** and clinical developments with antisense oligonucleotide-based delivery
- Rationalise the design criteria of **multi-functional nanoparticle** carriers
- Gain new insight of optimising **mRNA** therapeutics and delivery methods
- Understand industry hurdles of **targeted** delivery beyond the **liver**

Chairs for 2016:

Ryszard Kole,
Distinguished Scientist,
Sarepta Therapeutics

Nagy Habib,
Professor of Surgery,
Chairman & Co-founder
Imperial College London,
MiNA Therapeutics

Featured Speakers:

Punit Seth,
Executive Director, **Isis Pharmaceuticals**

Mustafa Diken,
Deputy Vice President, Immunotherapies and Preclinical Research, **BioNTech AG**

Christian Schetter,
Chief Executive Office and Managing Director, **Rigontech GmbH**

Cristianne Rijcken,
CSO, **Cristal Therapeutics**

Heinrich Haas,
Vice President RNA Formulation & Drug Delivery, **BioNTech AG**

Brian Kelly,
Associate, **Covington & Burling LLP**

Sponsored by

ACD
Advanced Cell Diagnostics

PLUS TWO INTERACTIVE HALF-DAY POST-CONFERENCE WORKSHOPS

Wednesday 17th February 2016, Holiday Inn Kensington Forum, London, UK

Workshop A

Next generation aptamer-conjugated RNA delivery in precision medicine

Workshop Leaders: **Dr. David Bunka,** Chief Technical Officer, and **Edward Barnes,** Research Scientist, **Aptamer Group Ltd.**

8.30am - 12.30pm

Workshop B

Strategies to optimize functional nanoparticle-mediated delivery of RNAi effectors to target tissues then progress to clinical studies

Workshop Leader: **Prof Andrew David Miller,** CSO, **GlobalAcorn Ltd**

12.30pm - 5.30pm

www.therapeutics-rna.com

Register online or fax your registration to +44 (0) 870 9090 712 or call +44 (0) 870 9090 711

CPD CERTIFIED
The CPD Certification Service

@SMIPHARM

RNA Therapeutics

Day One | Monday 15th February 2016

8.30 **Registration & Coffee**

9.00 **Chairman's Opening Remarks**
Ryszard Kole, Distinguished Scientist, **Sarepta Therapeutics**

Furthering the Role of Delivery Systems in RNA Therapeutics

OPENING ADDRESS

9.10 **Pre-clinical and clinical experience with oligonucleotide drugs**
Oligonucleotides and siRNAs went through ups and downs during their development as RNA therapeutics. Since the marketing of Fomivirsen for CMV retinitis, Kynamro (mipomersen) as a treatment for hypercholesterolemia adds only a second oligonucleotide to the existing pharmacopeia. It is anticipated that two new oligonucleotide drugs for the treatment of Duchenne muscular dystrophy will soon be evaluated by FDA.

Ryszard Kole, Distinguished Scientist, **Sarepta Therapeutics**

9.50 **A new class of RNA therapeutics – RIG-I**
- Introduction of basic technology and aspects of the MoA
- Discussion of preclinical results and development opportunities
- Introduction of Rigontec's lead compound
- Combinatorial approaches to boost immune response and tumor responses


Christian Schetter, Chief Executive Office and Managing Director, **Rigontech GmbH**

10.30 **Morning Coffee**

11.00 **Single copy detection of specific transcripts in the tissue**
RNAi related RNAscope applications:
- Tissue specific assessment of efficacy and validation of RNAi approaches
- Rapid Biomarker development and validation all the way to CDx
- Drug safety aspects and off-target effects in tissue samples
- Transfer from cell culture to animal models to clinical


Dr. Kai Wilkens, Senior Director Europe, **Advanced Cell Diagnostics**

11.40 **RNA vaccines development and optimising mRNA therapeutics and delivery**
- Clinical trial development
- Optimising mRNA for specific immune response
- Reviewing preclinical data of immune responses


Mustafa Diken, Deputy Vice President, Immunotherapies and Preclinical Research, **BioNTech AG**

12.20 **Networking Lunch**

1.30 **Keynote address: Targeted delivery of antisense oligonucleotides through ligand-conjugate chemistry**
- Targeted delivery to the liver
- Challenges of targeted delivery beyond the liver
- Characteristics of ligand receptor systems for successful delivery of oligonucleotide therapeutics
- Clinical trial progresses and future perspectives


Punit Seth, Executive Director, **Isis Pharmaceuticals**

Enabling RNA Therapeutics in Treatment

2.10 **Modulating gene expression with oligonucleotides that have a modifiable pharmacologic profile**
- Assessment of unassisted/gymnotic in vitro and in vivo delivery
- Design strategies modulating pharmacokinetics and improving the therapeutic window

Jürgen Soutschek, Biotech Consultant

2.50 **Keynote Address: Rational design including functionalisation of nanoparticles for improved cell delivery**
- Rationalising the design criteria of multi-functional nanoparticle carriers for various applications
- Assuring an initial biological stability of nanoparticles while still being biodegradable and non-toxic in the long
- Active targeting for increased cellular selectivity


Cristianne Rijcken, CSO, **Cristal Therapeutics**

3.30 **Afternoon Tea**

4.00 **Biotech 2.0 – Medical Revolution Through Natural mRNA**
- mRNA as a "data carrier" for a healthy message
- mRNA technology platform – vaccines (RNActive®) & therapeutics (RNArt®)
- Pre-clinical and clinical results

Nigel Horscroft, Director, Alliance Management, **Curevac**

4.40 **Regulating nano therapeutics and commercial interests**

Regulatory Insight

- Nanomedicine or device: Challenging the regulatory pathway and status
- Developing risk management plans: Appropriate governance and special considerations for data and safety
- Determining the intended use and therapeutic side effects: How are liabilities treated?
- Companion diagnostics and monitoring treatment effects


Brian Kelly, Associate, **Covington & Burling LLP**

5.20 **Panel discussion: Pushing towards and clinical success and market authorisation**
- Comparative review of delivery systems and striving beyond the liver
- Addressing liabilities and side effects of nanoparticles
- Advancements and insights in by-passing physiological barriers between the route of administration to the target
- Balancing ethical concerns and regulatory challenges on the road to market
- How can the regulatory framework be improved to adapt with the evolution of nanotechnology?

Panel leader: Nagy Habib, Professor of Surgery, Chairman and Co-founder, **Imperial College London, MiNa Therapeutics**
Panelists: Christian Schetter, Chief Executive Office and Managing Director, **Rigontech GmbH**
Ryszard Kole, Distinguished Scientist, **Sarepta Therapeutics**
Christian Schetter, Chief Executive Office and Managing Director, **Rigontech GmbH**
Jurgen Soutschek, Biotech Consultant
Nigel Horscroft, Director, **Alliance Management, Curevac**

6.00 **Chairman's Closing Remarks and Close of Day One**

Sponsored by

Advanced Cell Diagnostics

Advanced Cell Diagnostics, Inc. (ACD) is a leader in the emerging field of molecular pathology, developing cell- and tissue-based diagnostic tests for personalized medicine. Based in the heart of Silicon Valley, ACD was founded and managed by experienced entrepreneurs in the life science industry. ACD's products and services are based on its proprietary RNAscope® Technology, the first multiplex fluorescent and chromogenic *in situ* hybridization platform capable of detecting and quantifying RNA biomarkers *in situ* at single molecule sensitivity. The company's technology overcomes critical hurdles in the identification and validation of biomarkers for companion diagnostics. ACD's RNAscope® platform provides a new way of localizing and measuring RNA *in situ* with exceptional levels of sensitivity, specificity, and the ability to multiplex. Turnaround time for a new assay is reduced to three weeks. **www.acdbio.com**

Official Media Partners

RNAbiology
scientific computing world
TRANSCRIPTION
Translation

Official Publications

SPONSORSHIP AND EXHIBITION OPPORTUNITIES SMi offer sponsorship, exhibition, advertising and branding packages, uniquely tailored to complement your company's marketing strategy. Prime networking opportunities exist to entertain, enhance and expand your client base within the context of an independent discussion specific to your industry. Should you wish to join the increasing number of companies benefiting from sponsoring our conferences please call:
Alia Malick on **+44 (0) 20 7827 6168** or email: **amalick@smi-online.co.uk**

8.30 **Registration & Coffee**

9.00 **Chairman's Opening Remarks**
Nagy Habib, Professor of Surgery, Chairman and Co-founder, **Imperial College London, MiNa Therapeutics**

Accelerated Approval and Sustainable Production

9.10 **OPENING ADDRESS: RNA activation with small activating RNA: Liver application**
- RNA activation using small oligonucleotide is an emerging technology that allows genetic up regulation and protein expression without the use of gene therapy. This new technique relies on the use of a double strand oligonucleotide that targets both the promoter and enhancer region and leads to increased expression of messenger RNA in most cells.
- We exploited this technique to up regulate a liver transcription factor, CEBPa. This is one of the master regulators of the hepatocytes increases hepatic differentiation. In vivo administration of saRNA CEBPa led to improvement in liver function in models of liver failure, as well as non-alcoholic steatohepatitis (NASH). This positive data will be the basis for the technique to be applied clinically.

Nagy Habib, Professor of Surgery, Chairman and Co-founder, **Imperial College London, MiNa Therapeutics**

9.50 **Keynote address: How does the functionalisation of nanoparticles improve cell delivery**
- Case study: Functionalised nanoparticles associated to oligonucleotides with antibodies and aptamers

Claude Paul Malvy, Co-head of Research Group: Chemical Vectorology of Anticancer Drugs, **Gustave Roussy Institute**

10.30 **Morning Coffee**

11.00 **Small interfering RNA in targeting eye conditions**
- Rational design to optimise biodistribution and pharmacokinetics
- Uncovering gene-specific silencing mechanism where small RNA molecules are complementary to direct protein degradation
- RNA pipeline and clinical progress

Covadonga Paneda, R&D Manager, **Sylentis**

11.00 **Time to consider Freedom to Operate?**
- What is Freedom to Operate?
- What are the risks?
- Types of patent infringement and defences available
- Mitigating the risks - patent transactions and offensive options

James Legg, UK and European Patent Attorney, **Boult Wade Tennant**

11.40 **Novel mRNA therapeutics: Development from basic research to clinical practice**
- Therapeutic approaches based on mRNA
- Optimization of the mRNA stability and activity
- Formulation and delivery strategies for mRNA
- Process development and GMP manufacturing of mRNA therapeutics
- Clinical applications

Heinrich Haas, Vice President RNA Formulation and Drug Delivery, **BioNTech RNA Pharmaceuticals GmbH**

12.20 **Networking Lunch**

1.30 **Are precision therapeutics approaches the answer to enable functional delivery of RNAi effectors beyond the liver?**
- What are the outstanding problems facing functional RNAi delivery in vivo?
- How might we control further the PK of RNAi effector delivery in vivo?
- What options do we have for more control of RNAi effector PD behaviour in vivo?

- Consideration of next steps and thoughts on clinical applicability

Andrew David Miller, CSO, **Global Acorn Ltd.**

2.50 **Panel discussion: Decentralising research and more collaboration**

- Identifying opportunistic areas of collaboration to accelerate clinical development and manufacture
- One size does not fit all: Combinatorial approaches in RNA therapeutics
- cGMP manufacture

Panel leader: Nagy Habib, Professor of Surgery, Chairman and Co-founder, **Imperial College London, MiNa Therapeutics**
Panellist: Andrew David Miller, CSO, **Global Acorn Ltd.**

3.30 **Afternoon Tea**

4.00 **Novel properties sought from chemically-modified oligonucleotide molecules**
- Methods of mRNA repair
- Methods for characterization of endocytosis and intracellular delivery
- Clinical trial progresses and future perspectives

C. I. Edvard Smith, Professor, **Karolinska Institute**

4.40 **Aptamers as delivery vehicles targeting tumour cells**
- Automated selection of cell targeted aptamers
- RNAi uses in literature with aptamers

David Bunka, Chief Technical Officer, **Aptamer Group**

5.20 **Chairman's Closing Remarks and Close of Day Two**

Supported by

Want to know how you can get involved? Interested in promoting your services to this market?
Contact Teri Arri, SMi Marketing on +44 (0) 207 827 6162 or email: tarri@smi-online.co.uk

your registration to +44 (0)870 9090 712 or call +44 (0)870 9090 711

HALF-DAY POST-CONFERENCE WORKSHOP A
Wednesday 17th February 2016
8.30am – 12.30pm
Holiday Inn Kensington Forum, London, UK

Next generation aptamer-conjugated RNA delivery in precision medicine

Workshop Leaders:
Dr. David Bunka, Chief Technical Officer and
Edward Barnes, Research Scientist,
Aptamer Group Ltd.

Overview of workshop;
This is a must-attend workshop for anyone actively involved or interested in tranformational aptamer technologies, and will add great value to further creativity and entrepreneurship to your projects. Aptamer technology is looking to dominate the market due to their lower cost of production, faster timeline to commercialisation, and their capacity for greater target specificity. This workshop will take you through customized developments and scalable importunities to maximise output.

Key benefits of attending:
- **Achieve** greater specificity not seen in antibodies
- **Learn** application platforms and define your end product
- **Understand** integrated technologies in diagnostics and target specificity
- **Create** a reasonable timeline from concept to market

Programme

8.30 **Registration and Coffee**

9.00 **Opening remarks and introductions**

9.10 **Session 1: Aptamer technology**
- Aptamer vs Antibodies; superiority of targeted binding and manufacture and stability
- Selection for target-bound oligonucleotides; Afifnity and satbility

9.50 **Session 2: Aptamer selection and modification processes**
- Achieving desirable properties of aptamers and the iterative cyle of modification
- Case study and evaluation

10.30 **Morning Coffee**

11.00 **Session 3: Combinatorial libraries in ligand design**
- Strategic designs and development of chemical libraries
- Isolating efficient aptamers for your use; Randomised sequence selections

11.40 **Session 4: Clinical applications and promising horizons**
- Immune response levels
- Future prospects

12.20 **Closing remarks**

12.30 **Close of workshop**

About the workshop host:
Dr. David Bunka holds a Ph.D. in Molecular Biology from the University of Leeds (2003) and is a Geneticist by training. David has spent the last 12 years developing and running an aptamer selection facility at the University of Leeds and has built up a solid international reputation in the field.

David has authored several aptamer papers in peer reviewed journals, including 2 invited review articles and a book chapter on aptamer technologies. David has developed over 150 aptamers against a wide variety of targets including; small molecule antibiotics, food contaminants, disease associated proteins, several cancer associated cell-lines, viruses and patient tissue samples. This required the development of a similar number of process variations in order to achieve this.

David has industry leading expertise in high throughput liquid handling robotics and automated aptamer selection, making him one of the most experienced people in this aspect of the field worldwide.

About the organisation:
The Aptamer group of companies focuses on the development of aptamer technologies. We develop nucleic acid aptamers for use in research & development, biomarker discovery, diagnostics or therapeutic developments. Aptamers are short ssDNA or ssRNA sequences that offer high-affinity and specificity to virtually all types of targets. Aptamers are versatile, cost effective and offer a complementary or alternative solution to antibodies. We have over 20 years combined expertise in the development of nucleic acid aptamers to peptides, proteins, cells, tissue samples, micro-organisms and small molecules.

HALF-DAY POST-CONFERENCE WORKSHOP B
Wednesday 17th February 2016
1.30pm – 5.30pm
Holiday Inn Kensington Forum, London, UK

Strategies to optimize functional nanoparticle–mediated delivery of RNAi effectors to target tissues then progress to clinical studies

Workshop Leader:
Prof. Andrew David Miller, CSO, **GlobalAcorn Ltd**

Overview of workshop:
The need for functional delivery of RNAi effectors to target cells is a primary problem that is holding back the field of RNAi therapeutics. Nanoparticles, molecular conjugates, and other delivery technologies have their place but are they really working well enough? If not, why not? And if they do appear to be working, what will it actually take to realize clinical success? This workshop is designed to help attendees appreciate the magnitude of the gap that must be bridged between academic-preclinial studies and the needs of the pharma industrial for success in RNAi therapeutics.

Key benefits of attending:
This workshop is a must-attend for all those puzzled, perplexed or confused about what it is really going to take to move from successful RNAi therapy experiments in preclinical models of human disease to effective implementation of RNAi type therapeutics in clinic.

Programme

1.30 **Registration**

2.00 **Opening remarks and introductions**

2.10 **Session 1: Functional RNAi delivery by nanoparticles**
- Problems
- Potential solutions

2.50 **Session 2: Manufacture of nanoparticles**
- Quality assurance
- Cost-efficient production

3.30 **Afternoon Tea**

4.00 **Session 3: Integrated development plans**
- Non-clinical studies
- Regulatory issues
- Clinical trials

4.40 **Session 4: Summarizing the challenges ahead**
- Use of appropriate delivery technologies
- Right clinical studies

5.20 **Closing remarks**

5.30 **Close of workshop**

About the workshop host:

Prof Miller is a leading chemist expert in the understanding and exploitation of molecular mechanisms in biology. Up until 2010, he was a at Imperial College London, where he was full Professor of Organic Chemistry and Chemical Biology since 2002, plus founding Director of the Imperial College Genetic Therapies Centre since 1998. From 2010 onwards, Prof Miller has been based in the UK at King's College London from where he has been developing his career as an academic entrepreneur, co-founding and helping to run GlobalAcorn Ltd since 2011.

About the organisation:
GlobalAcorn is an innovative clinical stage biopharmaceutical company that specialises in Precision Therapeutics Approaches (PTAs) for the diagnosis and treatment of chronic diseases. These PTAs are based upon the company's expertise in targeted nanomedicine imaging, delivery platform technologies and target specific design of active pharmaceutical ingredients (APIs). Our novel PTAs mobilise and concentrate APIs at disease target site(s) thereby maximising therapeutic benefits while minimising adverse effects. GlobalAcorn currently operates across several channels, with a lead candidate in oncology-cancer nanomedicine.

RNA THERAPEUTICS

Conference: Monday 15th & Tuesday 16th February 2016, Holiday Inn Kensington Forum, London, UK
Workshops: Wednesday 17th February 2016, London, UK

4 WAYS TO REGISTER

www.therapeutics-rna.com

FAX your booking form to +44 (0) 870 9090 712
PHONE on +44 (0) 870 9090 711

POST your booking form to: Events Team, SMi Group Ltd, 2nd Floor South, Harling House, 47-51 Great Suffolk Street, London, SE1 0BS, UK

Unique Reference Number	
Our Reference	LVP-162

DELEGATE DETAILS

Please complete fully and clearly in capital letters. Please photocopy for additional delegates.

Title: **Forename:**

Surname:

Job Title:

Department/Division:

Company/Organisation:

Email:

Company VAT Number:

Address:

Town/City:

Post/Zip Code: **Country:**

Direct Tel: **Direct Fax:**

Mobile:

Switchboard:

Signature: **Date:**

I agree to be bound by SMi's Terms and Conditions of Booking.

ACCOUNTS DEPT

Title: **Forename:**

Surname:

Email:

Address (if different from above):

Town/City:

Post/Zip Code: **Country:**

Direct Tel: **Direct Fax:**

VENUE Holiday Inn Kensington Forum, 97 Cromwell Rd, London SW7 4DN, UK

☐ **Please contact me to book my hotel**
Alternatively call us on +44 (0) 870 9090 711,
email: events@smi-online.co.uk or fax +44 (0) 870 9090 712

Terms and Conditions of Booking

Payment: If payment is not made at the time of booking, then an invoice will be issued and must be paid immediately and prior to the start of the event. If payment has not been received then credit card details will be requested and payment taken before entry to the event. Bookings within 7 days of event require payment on booking. Access to the Document Portal will not be given until payment has been received.

Substitutions/Name Changes: If you are unable to attend you may nominate, in writing, another delegate to take your place at any time prior to the start of the event. Two or more delegates may not 'share' a place at an event. Please make separate bookings for each delegate.

Cancellation: If you wish to cancel your attendance at an event and you are unable to send a substitute, then we will refund/credit 50% of the due fee less a £50 administration charge, providing that cancellation is made in writing and received at least 28 days prior to the start of the event. Regretfully cancellation after this time cannot be accepted. We will however provide the conferences documentation via the Document Portal to any delegate who has paid but is unable to attend for any reason. Due to the interactive nature of the Briefings we are not normally able to provide documentation in these circumstances. We cannot accept cancellations of orders placed for Documentation or the Document Portal as these are reproduced specifically to order. If we have to cancel the event for any reason, then we will make a full refund immediately, but disclaim any further liability.

Alterations: It may become necessary for us to make alterations to the content, speakers, timing, venue or date of the event compared to the advertised programme.

Data Protection: The SMi Group gathers personal data in accordance with the UK Data Protection Act 1998 and we may use this to contact you by telephone, fax, post or email to tell you about other products and services. Unless you tick here ☐ we may also share your data with third parties offering complementary products or services. If you have any queries or want to update any of the data that we hold then please contact our Database Manager databasemanager@smi-online.co.uk or visit our website www.smi-online.co.uk/updates quoting the URN as detailed above your address on the attached letter.

EARLY BIRD DISCOUNT

- ☐ **Book by 30th October 2015 to receive £400 off the conference price**
- ☐ **Book by 30th November 2015 to receive £200 off the conference price**
- ☐ **Book by 18th December 2015 to receive £100 off the conference price**

CONFERENCE PRICES

I would like to attend: (Please tick as appropriate)	**Fee**		**Total**
☐ Conference & 2 Workshops	**£2697.00**	**+ VAT**	**£3236.40**
☐ Conference & 1 Workshop AM ☐ PM ☐	**£2098.00**	**+ VAT**	**£2517.60**
☐ Conference only	**£1499.00**	**+ VAT**	**£1798.80**
☐ 1 Workshop only AM ☐ PM ☐	**£599.00**	**+ VAT**	**£718.80**
☐ 2 Workshops	**£1198.00**	**+ VAT**	**£1437.60**

PROMOTIONAL LITERATURE DISTRIBUTION

☐ Distribution of your company's promotional literature to all conference attendees	**£999.00**	**+ VAT**	**£1198.80**

The conference fee includes refreshments, lunch, conference papers, and access to the Document Portal. Presentations that are available for download will be subject to distribution rights by speakers. Please note that some presentations may not be available for download. Access information for the document portal will be sent to the e-mail address provided during registration. Details are sent within 24 hours post conference.

DOCUMENTATION

I cannot attend but would like to purchase access to the following Document Portal/paper copy documentation

	Price		**Total**
☐ Access to the conference documentation on the Document Portal	**£499.00**	**+ VAT**	**£598.80**
☐ The Conference Presentations – paper copy (or only £300 if ordered with the Document Portal)	**£499.00**	**-**	**£499.00**

PAYMENT

Payment must be made to **SMi Group Ltd**, and received before the event, by one of the following methods **quoting reference P-162 and the delegate's name. Bookings made within 7 days of the event require payment on booking, methods of payment are below. Please indicate method of payment:**

- ☐ **UK BACS** Sort Code **300009**, Account **00936418**
- ☐ **Wire Transfer** Lloyds TSB Bank plc, 39 Threadneedle Street, London, EC2R 8AU
  Swift (BIC): **LOYDGB21013,** Account **00936418**
  IBAN **GB48 LOYD 3000 0900 9364 18**
- ☐ **Cheque** We can only accept Sterling cheques drawn on a UK bank.
- ☐ **Credit Card** ☐ Visa ☐ MasterCard ☐ American Express

All credit card payments will be subject to standard credit card charges.

Card No: ☐☐☐☐ ☐☐☐☐ ☐☐☐☐ ☐☐☐☐

Valid From ☐☐/☐☐ Expiry Date ☐☐/☐☐

CVV Number ☐☐☐☐ 3 digit security on reverse of card, 4 digits for AMEX card

Cardholder's Name:

Signature: **Date:**

I agree to be bound by SMi's Terms and Conditions of Booking.

Card Billing Address (If different from above):

VAT

VAT at 20% is charged on the attendance fees for all delegates. VAT is also charged on Document portal and literature distribution for all UK customers and for those EU Customers not supplying a registration number for their own country here.

If you have any further queries please call the Events Team on tel +44 (0) 870 9090 711 or you can email events@smi-online.co.uk