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Delivering RNAi-Based Therapeutics & Diagnostics Challenges & Opportunities

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Abstracts

This cutting edge & insightful report can be used to interpret & assess the potential of RNAi-based medicines. It provides opinions & market projections to:

assess the commercial potential of RNAi-based therapies in all of the key therapeutic areas: cancer, CNS disorders, viral infectious diseases, ocular diseases, cardiovascular, metabolic and other conditions;

identify key pharma & delivery specialists advancing RNAi-based therapeutics & diagnostics;

gain an in-depth understanding of the technological & delivery issues which face companies developing RNAi-based therapies;

evaluate the options available for chemically and structurally modifying RNAi silencing agents and delivering them to target tissues now & in the future;

discover which companies are tackling efficacy and safety issues & are in a prime position to exploit new opportunities;

analyze how the market will evolve over the next decade highlighting key trends, opportunities & challenges.

KEY FINDINGS:

Since the 1990s scientists across the world have begun to harness the power of

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RNA interference (RNAi) to silence genes in a multitude of diseases. However, many challenges face those aiming to develop RNAi-based therapies including targeted delivery, the design and efficacy of gene silencing agents and the minimization of off-target and immunogenic effects.

Today there are over 100 RNAi-based projects under investigation to treat autoimmune and inflammatory diseases, cancer, cardiovascular disease, CNS disorders, metabolic, ocular conditions, and viral infectious diseases. The majority of these projects are in the earliest discovery stages (36%) or in preclinical studies (54%). Of the projects that have reached clinical trials, only a handful have passed initial Phase 1/2 testing, with a total of three being investigated in Phase 2 studies.

In 1H09 two late-stage ocular RNAi-based therapies, Opko Health's bevasiranib and Allergan's AGN-745, were terminated in late-stage clinical trials after the drugs failed to meet a key efficacy endpoint. This is a major set back for market and raises questions regarding the potential of unformulated siRNAs as therapeutics.

This market continues to be driven by a greater understanding of the genetic component of a broad range of diseases and the development of targeted carrier systems to ensure efficient local, systemic and oral delivery of the RNAi silencing agents.

Innovative structures and chemical modifications are being explored to improve stability, efficacy and delivery of RNAi-based therapeutics and reduce off-target effects. In addition, a number of novel RNAi silencing agents have been identified with therapeutic and diagnostic potential including dicer substrate RNA (disRNA) and anti-miRNAs.

Despite these challenges and others, such as the complexities of regulation and intellectual property, the field retains enormous potential to diagnose and treat diseases with high unmet medical need. Specialists and academics are working hard to find ways around these hurdles in order to deliver a new generation of RNAi-based therapeutics and diagnostics.

The market for RNAi-based therapeutics is forecast to grow from 2013 onwards, as the first products enter the marketplace, to generate sales in excess of US$2.9 billion by 2020. The first siRNA based therapeutics will capitalize on the

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demand to treat viral infections and ocular conditions and in the longer term companies will be able to target niche areas of high unmet clinical need such as cancer, cardiovascular disease, metabolic disorders, inflammatory and neurological conditions.

Introduction

“Several novel RNAi-based therapeutics are expected to be launched in the next ten years as companies have made significant advances in identifying, characterising, designing and delivering gene silencing agents to a variety of target tissues. A plethora of RNAi start up companies have emerged during the last few years as the field hots up and large pharma such as Novartis, Merck, Pfizer and Roche begin to make strategic acquisitions and collaborations with market leaders. The results of a number of Phase 2 clinical trials may help to quell researchers’ fears regarding the efficacy of RNAi-based therapeutics and drive investment for the future.”

Dr Cheryl Barton

Over the last decade, tremendous advances have been made in the field of RNAinterference (RNAi), a naturally occurring mechanism for gene regulation. Researchers have begun to unravel the underlying mechanisms of gene silencing and along the way a number of new gene silencing agents have been uncovered such as microRNAs.

Whilst RNAi has become a useful tool for understanding the function of specific genes and a means of identifying new targets for small molecule intervention, many scientists have harnessed its power to develop RNAi-based therapeutics, which can treat and in some cases prevent disease and have expanded the repertoire of targets previously deemed ‘undruggable’.

The development of RNA-based therapeutics has faced many challenges, namely stability, efficacy, potency and delivery. Whilst many of these hurdles have been overcome, delivery has played a key role in tempering the speed at which RNAi therapeutics have progressed into the clinic. The first generation RNAi therapeutics to reach the clinic are chemically unmodified, naked short interfering RNA (siRNA) and although these have proved the concept that gene silencing has potential to regulate disease specific genes, the jury is still out regarding their therapeutic mechanism of action and long-term safety.

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Meanwhile a plethora of delivery platforms have evolved to improve targeted delivery, duration of action, stability and to reduce the off-target effects of RNAi agents and this has also helped to expand their ease of administration from localized and topical formulations to systemic and more recently oral delivery.

In addition, a second generation of RNAi therapeutics, dicer substrate RNA (disRNA), have been discovered that act higher up the RNAi pathway than siRNAs and use endogenous enzymes to process them into siRNAs. Interestingly, this new class of RNA-based therapeutics appear to be more potent and have a longer duration of action than their siRNA counterparts.

However, this field is still in its infancy and the full potential of RNA-based therapeutics has yet to be realized. To date, no RNAi-based therapeutic has been approved, although several products are in clinical development.

EIGHT QUESTIONS THIS NOTE ANSWERS:

1.Which companies & academic institutes are actively involved in RNAi-based therapeutic research?
2.What are the key areas of therapeutic focus for RNAi-based therapeutics in the near & long-term?
3.What are the key obstacles companies & delivery technologists need to overcome to commercialize RNAi-derived therapies?
4.How will delivery technologies drive the development of innovative RNAi-based therapies in the future?
5.Which RNAi technologies & agents are likely to win in the near-term & long-term, & why?
6.Where are the market opportunities now & in the future?
7.When are RNAi-based medicines likely to reach the market?
8.What do we predict will be the value of the RNAi-based therapeutics by 2015 & 2020?

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COMPANIES MENTIONED:

RNAi therapeutic companies: AiRNA Pharmaceuticals, Allergan, Alnylam Pharmaceuticals, Benitec, BioCandell Therapeutics, Calando Pharmaceuticals, Cenix BioScience, Cequent Pharmaceuticals, CopernicusTherapeutics, Dicerna Pharmaceuticals, Expression Genetics Corporation, General Research Laboratory, Intradigm Corporation, MDRNA, Mirna Therapeutics, MiRagen Therapeutics, Nitto Denko Technical Corporation, Opko Health, Oxford BioMedica, Polyplus Transfection, Quark Pharmaceuticals, Regulus Therapeutics, RXi Therapeutics, Santaris Pharma, Senesco Technologies, Silence Therapeutics, Siranoamics, siRNAsense A/S, Sylentis Pharmaceuticals, Tacere Therapeutics, Targeted Genetics Corporation & Tekmira Pharmaceuticals Corporation.

Antisense therapeutic companies: Isis Pharmaceuticals, Lorus Therapeutics & Santaris Pharma.

Pharmaceuticals & biotechnology companies: AstraZeneca, Bristol-Myers Squibb, Biogen Idec, Cubist Pharmaceuticals, Eli Lilly, Enzon, Genzyme, Kyowa Hakko Kirin, Merck & Co., Novartis, Oncolys BioPharma, Pfizer, Roche & Takeda.

RNAi diagnostic companies: Asuragen Diagnostics, Exiqon Diagnostics & Rosetta Genomics

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