



**Case studies on rare disease therapies development
A laboratory of ideas for European innovation
leadership – Alnylam perspective**

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RNAi Therapeutics: A Timeline

From Observation to Nobel Prize to Innovative Medicines in ~3 Decades



First scientific report of RNAi phenomenon in which Napoli and Jorgensen report that violet petunias turned white instead of a deeper violet¹

letters to nature

Potent and specific genetic interference by double-stranded RNA in *Caenorhabditis elegans*

Andrew Fire¹, SiQun Xu¹, Mary K. Montgomery¹, Steven A. Kostas¹, Samuel E. Driver¹ & Craig C. Mello²
¹ Carnegie Institution of Washington, Department of Embryology, 115 West University Parkway, Baltimore, Maryland 21210, USA
² Biology Graduate Program, Johns Hopkins University, 3400 North Charles Street, Baltimore, Maryland 21218, USA
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Fire and Mello published a paper that reported a potent gene silencing effect in worms and coined the term RNA interference²



Anylam founded with a core focus on developing RNAi therapeutics



Fire and Mello awarded the 2006 Nobel Prize in Physiology or Medicine

First Anylam RNAi APPROVED in U.S, EU, Canada, Switzerland and Japan

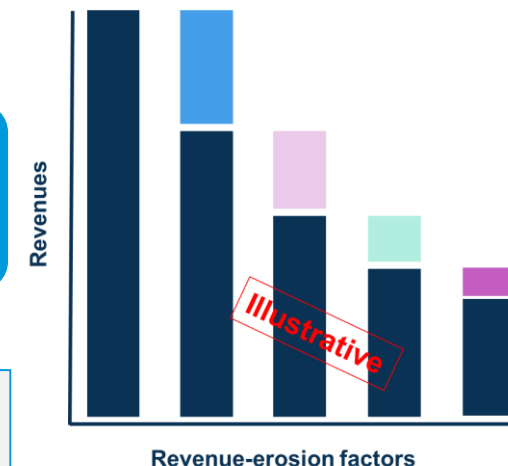
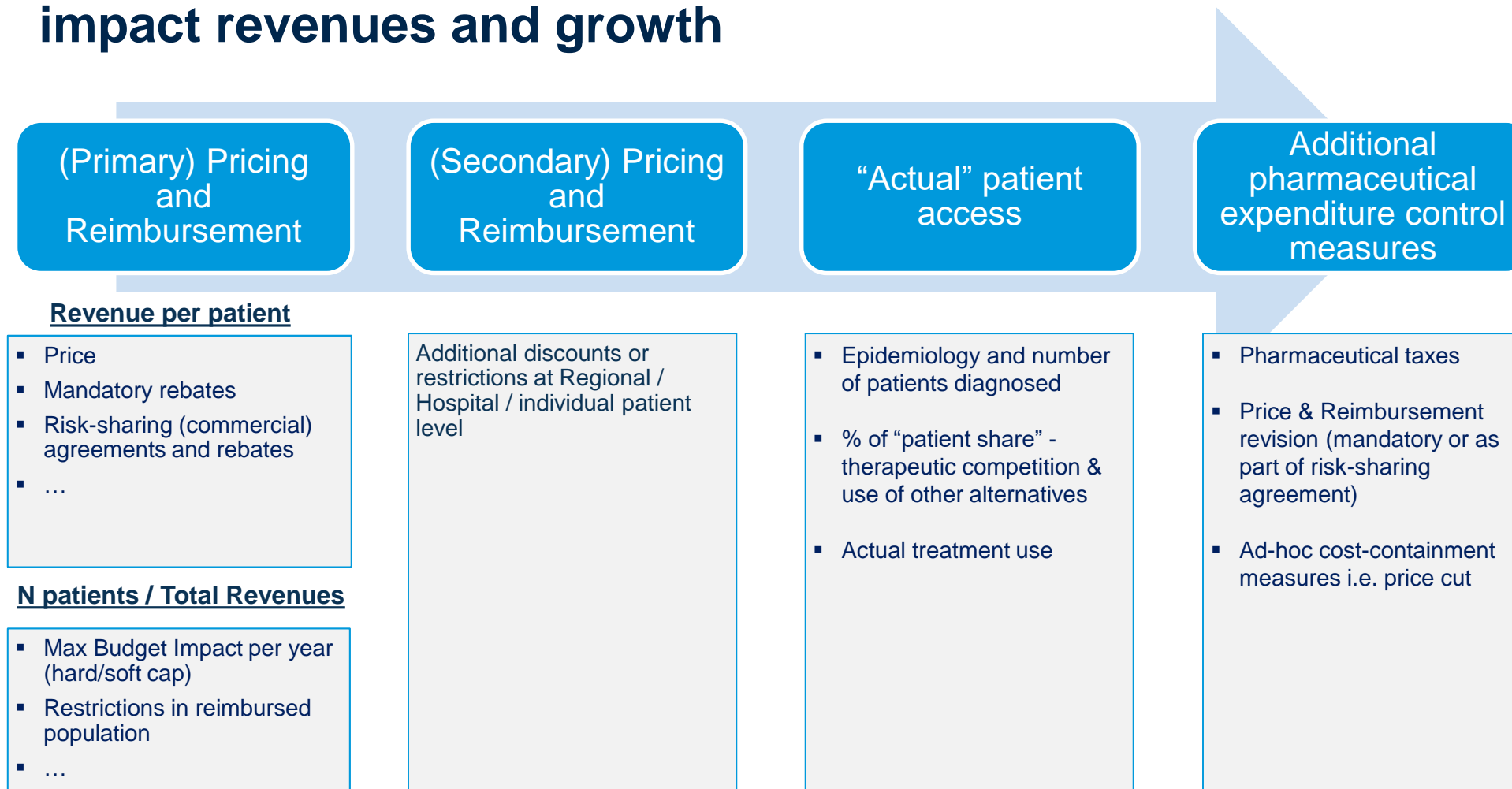
Second Anylam RNAi APPROVED APPROVED in U.S.

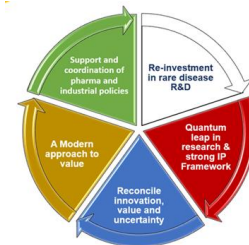
APPROVED in E.U.

Third Anylam RNAi APPROVED in EU



Patient Access does not stop at reimbursement, and multiple factors may impact revenues and growth





Leveraging Pricing and Reimbursement for holistic industrial and public health policy – new models are merging

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Press release

New heart disease drug to be made available for NHS patients

The government is collaborating with pharmaceutical company Novartis to launch a clinical trial for new cholesterol treatment.

apm news

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https://www.apmnews.com/story.php?objet=354076&idmail=0.0Q4xQ035b7LrDKvH8QwA4CmVhZrDdQ9rHzCk-BIpgErGV5IHZ0aeLXQuzDCrDkRkGbsUwB_sok4gM0-5vOyrWCRHWFZ50aTj_xEc3y0RkXQvWxgAdkxyCR0mLT1ma5f0e45XWxkQvhu0Y25APCSA2Dye0U7J02H7x_xjandDrdj5m2B6BU51X4gM_8MPeFxdRfwaPOYH_gC-yq3VA_8YAO0UjKcUcRBLXqgAKLU15Gcbi0_1aj

DEPÊCHE DU 17/07/2020

Des laboratoires spécialistes des maladies rares proposent un mécanisme pour faciliter l'accès au marché

Mots-clés : #produits de santé #ministères #biotech #financement d'entreprise #maladies rares #accès au marché #prix #ATU #médicaments #ministère-recherche #ministère-santé #Bercy #Matignon

- Reward innovation & value
- Provide room for growth & revenues for fast-growing biotech companies investing in setting-up their local operations
- Create safeguard to anticipate and mitigate any uncertainty on the value of the product, or the financial impact related to its introduction
- Incentivize reinvestment of revenues into local R&D or Public Private Partnership

From minimizing cost to maximizing ROI of pharmaceutical spending on innovative drugs

Getting there is a shared responsibility, and we need a new Social Contract between Biotech and Society

70 leaders from Biotech and VCs ready to find game-changing solutions



Life science leaders sign new 'Biotech Social Pact' for Europe

September 30, 2020

The signatories of a new [Biotech Social Pact](#) with Europe and patients explain why healthcare calls for a new social contract, not a pharmaceutical strategy.

As Europe continues its efforts to battle the Covid-19 pandemic, it is rightfully focusing its attention on the longer-term implications of this crisis. One common thread is clearly emerging from EU and national discussions: to reinforce Europe's "strategic autonomy" and "technological sovereignty" in key industrial sectors.

This is a key theme of the EU recovery plan but also the EU4Health and expected new "Pharmaceutical Strategy" that will "aim to ensure Europe's supply of safe and affordable medicines to meet patients' needs and support the European

Medium

A new European Biotech Social Pact with Europe and Patients



We the undersigned leaders in the biotechnology industry and life science investors in Europe and in the US recognize that at this pivotal moment in the history of our industry, we are at the dawn of a golden age of medicine. We will soon have the ability to treat and cure diseases which have been untreatable and incurable.

Politico

Biotech leaders pledge to prioritize patients, not profits

-- By Ashleigh Furlong
9/29/20, 9:37 AM CET | [View in your browser](#)

Over 60 leaders of biotech companies have promised to put patients' interests first in a "social pact" that commits to the ethical use of incentives and reinvestment in Europe.

Pharmaphorum

Life science leaders sign new 'Biotech Social Pact' for Europe

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NRC

N.B. Het kan zijn dat elementen ontbreken aan deze printversie.

Leiders in biotech beloven 'ethische' omgang met prijzen en patenten

Dure medicijnen Zestig directeuren uit de biotechsector beloven 'integer' te zullen handelen als zij nieuwe medicijnen op de markt brengen.



Thank you