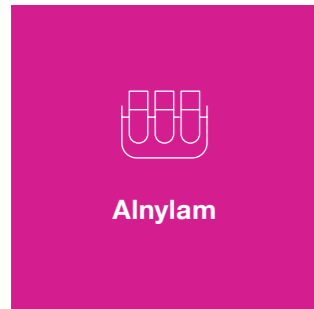


# Anylam

RNAi therapeutics

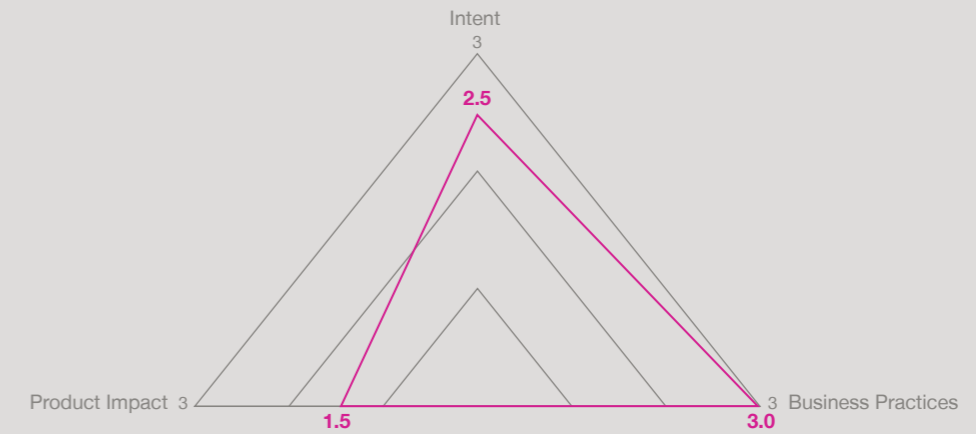


Anylam is attempting to develop innovative medicines known as RNAi – a natural process of gene silencing. RNAi prevents the production of disease-causing proteins. Anylam’s pipeline is focused in four strategic therapeutic areas: Genetic Medicines, Cardio-Metabolic Disease, Hepatic Infectious Disease and Central Nervous System.

## Investment Case

<b>What challenge is the Company tackling?</b>	Rare and chronic diseases impact quality and quantity of life, can cause significant collateral damage to the rest of the body and are expensive to treat. The potential benefit to the patient of RNAi therapy is huge; you will still have a disease at the genetic level but by taking a drug you do not have to live with the consequences or symptoms.
<b>Is the Company's offering sufficiently different and better than the status quo?</b>	Given how nascent this opportunity is, it is pretty challenging to define. Suggested competitive advantages include its innovative approach to science and to some extent its delivery platform, known as GalNAc. It is worth noting that competitor Ionis has licensed the GalNAc delivery mechanism. The company’s approach is undoubtedly linked to its culture which aims to excel in research and science as a primary goal.
<b>Is there a committed management team? What have they done before?</b>	The company’s mission is described as being founded on a revolutionary vision to harness RNAi to improve human health. Anylam was founded in MIT in 2002 by three scientists: Phillip Sharp who won the Nobel prize in 1993 for work on gene splicing and was also a founder of Biogen and still sits on the Board; Paul Schimmel who is now at Scripps Research Institute and on the Board and Thomas Tuschl a German expert on RNA. The current CEO John Maraganore joined at the foundation of the company in 2002 with 25 years of biotech experience. He has a PhD in biochemistry and molecular biology. The management team have been consistent in describing the science-based culture of the company.
<b>How does the Company treat stakeholders?</b>	It is clear that Anylam’s patient focus is significant. In terms of employees, they have been on the Boston Globe’s top places to work list for the last 3 years and their CEO has a 96% approval rating on Glassdoor. Further to this the company operates an employee stock purchase plan to facilitate alignment of personal and company goals. There is good gender diversity at Board and management level, including a female COO which is an unusual occurrence. There are a range of national and ethnic backgrounds in the management team which suggests that they do what they say with regard to diversity and inclusion – the CEO signed a protest letter regarding Trump’s travel ban and has been outspoken about the criticality of diversity and the importance of immigration to the biotech sector.
<b>Is there a potential to create a profitable and defensible business?</b>	The potential opportunity here is significant given a large number of human diseases are caused by abnormal proteins. It has a very rich pipeline across four strategic therapeutic areas. That being said, predicting which of their other drugs might succeed is virtually impossible; and knowing what other areas of protein deficiency the company may be able to address is beyond us. However, with 30 million Americans suffering from 7,000 rare diseases there is a huge market opportunity from this starting point.
<b>Is the business attractively valued?</b>	This an early stage company with large opportunities for growth. Management are clear that Anylam will remain in heavy investment mode for the foreseeable future (recently capital has been mostly deployed in R&D and the acquisition of intellectual property assets), with the intention of generating substantial value over the next 10 – 20 years. The future level of profitability is largely dependent on the scale of individual drug successes, which leaves wide margins of possibilities. If all of its phase 2 and 3 drugs succeed, then Anylam could have 6 drugs on the market by 2023. Longer term there is the opportunity to expand into other complex diseases. In many ways, analysing the probability of Anylam doubling in value over 5 years does not capture the opportunity of the company which is likely to be realised over a much longer timeframe.

## Impact Analysis



### Product Impact

Anylam are the ultimate niche product in that they don’t actually have one! The score reflects the potential, which is enormous, and the reasonable expectation that something will come of it, even if just in the rare diseases they are currently pursuing. This score could increase considerably in time.

### Intent

In the context of the portfolio Anylam fit into the category of one of the great innovators. Their work on RNAi has the potential to create a whole new class of drug which could have implications far beyond their own organisation. Ground breaking science is very strongly embedded in their structures through their management team, Board and Scientific Advisory Board.

### Business Practices

It is quite difficult to score a company whose interactions are fairly limited based on its current research focus. However, as well as ticking boxes across the range of ESG issues that are applicable, on a couple of occasions Anylam has stuck its neck out on issues that are important in the industry such as diversity and pricing.

	Relevant Targets	Relevant Products
	<p><b>Target 3.3</b> By 2030, end the epidemics of AIDS, tuberculosis, malaria and neglected tropical diseases and combat hepatitis, water-borne diseases and other communicable diseases.</p> <p><b>Target 3.4</b> By 2030, reduce by one third premature mortality from non-communicable diseases through prevention and treatment and promote mental health and well being.</p>	<p>A pipeline of potentially transformative medicines in areas of high unmet medical need/ rare diseases: Cardio- Metabolic Diseases and Hepatic Infectious Diseases, with product candidates designed to address the major global health challenges of hepatic infectious diseases, beginning with hepatitis B and hepatitis D viral infections. Anylam Investigational RNAi Therapeutics demonstrated potential in clinical trials to achieve robust clinical activity with up to 99% target gene knockdown in some cases.</p>

**This presentation is intended solely for the use of professional investors and should not be relied upon by any other person. It is not intended for use by retail clients.**

---

## IMPORTANT INFORMATION AND RISK FACTORS

---

Baillie Gifford & Co and Baillie Gifford & Co Limited are authorised and regulated by the Financial Conduct Authority (FCA). Baillie Gifford & Co Limited is an Authorised Corporate Director of OEICs’.

Baillie Gifford Overseas Limited provides investment management and advisory services to non-UK Professional/Institutional clients only. Baillie Gifford Overseas Limited is wholly owned by Baillie Gifford & Co. Baillie Gifford & Co and Baillie Gifford Overseas Limited are authorised and regulated by the FCA in the UK.

Baillie Gifford Investment Management (Europe) Limited provides investment management and advisory services to European (excluding UK) clients. It was incorporated in Ireland in May 2018 and is authorised by the Central Bank of Ireland. Through its MiFID passport, it has established Baillie Gifford Investment Management (Europe) Limited (Frankfurt Branch) to market its investment management and advisory services and distribute Baillie Gifford Worldwide Funds plc in Germany. Similarly, it has established Baillie Gifford Investment Management (Europe) Limited (Amsterdam Branch) to market its investment management and advisory services and distribute Baillie Gifford Worldwide Funds plc in The Netherlands.

Baillie Gifford Investment Management (Europe) Limited also has a representative office in Zurich, Switzerland pursuant to Art. 58 of the Federal Act on Financial Institutions (“FinIA”). It does not constitute a branch and therefore does not have authority to commit Baillie Gifford Investment Management (Europe) Limited. It is the intention to ask for the authorisation by the Swiss Financial Market Supervisory Authority (FINMA) to maintain this representative office of a foreign asset manager of collective assets in Switzerland pursuant to the applicable transitional provisions of FinIA.

Baillie Gifford Investment Management (Europe) Limited is a wholly owned subsidiary of Baillie Gifford Overseas Limited, which is wholly owned by Baillie Gifford & Co. Baillie Gifford Overseas Limited and Baillie Gifford & Co are authorised and regulated in the UK by the Financial Conduct Authority.

Persons resident or domiciled outwith the UK should consult with their professional advisers as to whether they require any governmental or other consents in order to enable them to invest, and with their tax advisers for advice relevant to their own particular circumstances.

### Important Information Hong Kong

Baillie Gifford Asia (Hong Kong) Limited 柏基亞洲(香港)有限公司 is wholly owned by Baillie Gifford Overseas Limited and holds a Type 1 and a Type 2 licence from the Securities & Futures Commission of Hong Kong to market and distribute Baillie Gifford’s range of collective investment schemes to professional investors in Hong Kong. Baillie Gifford Asia (Hong

Kong) Limited 柏基亞洲(香港)有限公司 can be contacted at Suites 2713-2715, Two International Finance Centre, 8 Finance Street, Central, Hong Kong, Telephone +852 3756 5700.

### Important Information Japan

Mitsubishi UFJ Baillie Gifford Asset Management Limited (“MUBGAM”) is a joint venture company between Mitsubishi UFJ Trust & Banking Corporation and Baillie Gifford Overseas Limited. MUBGAM is authorised and regulated by the Financial Conduct Authority.

### Important Information Australia

Baillie Gifford Overseas Limited (ARBN 118 567 178) is registered as a foreign company under the Corporations Act 2001 (Cth) and holds Foreign Australian Financial Services Licence No 528911. This material is provided to you on the basis that you are a “wholesale client” within the meaning of section 761G of the Corporations Act 2001 (Cth) (“Corporations Act”). Please advise Baillie Gifford Overseas Limited immediately if you are not a wholesale client. In no circumstances may this document be made available to a “retail client” within the meaning of section 761G of the Corporations Act. This material contains general information only. It does not take into account any person’s objectives, financial situation or needs.

### Important Information South Africa

Baillie Gifford Overseas is registered as a Foreign Financial Services Provider with the Financial Sector Conduct Authority in South Africa.

### Important Information North America

Baillie Gifford International LLC is wholly owned by Baillie Gifford Overseas Limited; it was formed in Delaware in 2005 and is registered with the SEC. It is the legal entity through which Baillie Gifford Overseas Limited provides client service and marketing functions in North America. Baillie Gifford Overseas Limited is registered with the SEC in the United States of America.

The Manager is not resident in Canada, its head office and principal place of business is in Edinburgh, Scotland. Baillie Gifford Overseas Limited is regulated in Canada as a portfolio manager and exempt market dealer with the Ontario Securities Commission (“OSC”). Its portfolio manager licence is currently passported into Alberta, Quebec, Saskatchewan, Manitoba and Newfoundland & Labrador whereas the exempt market dealer licence is passported across all Canadian provinces and territories. Baillie Gifford International LLC is regulated by the OSC as an exempt market and its licence

is passported across all Canadian provinces and territories. Baillie Gifford Investment Management (Europe) Limited (“BGE”) relies on the International Investment Fund Manager Exemption in the provinces of Ontario and Quebec.

### Important Information South Korea

Baillie Gifford Overseas Limited is licensed with the Financial Services Commission in South Korea as a cross border Discretionary Investment Manager and Non-discretionary Investment Adviser.

### Important Information Oman

Baillie Gifford Overseas Limited (“BGO”) neither has a registered business presence nor a representative office in Oman and does not undertake banking business or provide financial services in Oman. Consequently, BGO is not regulated by either the Central Bank of Oman or Oman’s Capital Market Authority. No authorization, licence or approval has been received from the Capital Market Authority of Oman or any other regulatory authority in Oman, to provide such advice or service within Oman. BGO does not solicit business in Oman and does not market, offer, sell or distribute any financial or investment products or services in Oman and no subscription to any securities, products or financial services may or will be consummated within Oman. The recipient of this document represents that it is a financial institution or a sophisticated investor (as described in Article 139 of the Executive Regulations of the Capital Market Law) and that its officers/employees have such experience in business and financial matters that they are capable of evaluating the merits and risks of investments.

### Important Information Qatar

The materials contained herein are not intended to constitute an offer or provision of investment management, investment and advisory services or other financial services under the laws of Qatar. The services have not been and will not be authorised by the Qatar Financial Markets Authority, the Qatar Financial Centre Regulatory Authority or the Qatar Central Bank in accordance with their regulations or any other regulations in Qatar.

### Important Information Israel

Baillie Gifford Overseas is not licensed under Israel’s Regulation of Investment Advising, Investment Marketing and Portfolio Management Law, 5755-1995 (the Advice Law) and does not carry insurance pursuant to the Advice Law. This document is only intended for those categories of Israeli residents who are qualified clients listed on the First Addendum to the Advice Law.

### Potential for Profit and Loss

All investment strategies have the potential for profit and loss. Past performance is not a guide to future returns. It should not be assumed that recommendations/transactions made in the future will be profitable or will equal performance of the securities mentioned.

This document contains information on investments which does not constitute independent research. Accordingly, it is not subject to the protections afforded to independent research and Baillie Gifford and its staff may have dealt in the investments concerned.

All information is sourced from Baillie Gifford & Co and is current unless otherwise stated.

The images used in this document are for illustrative purposes only

### Financial Intermediaries

This document is suitable for use of financial intermediaries. Financial intermediaries are solely responsible for any further distribution and Baillie Gifford takes no responsibility for the reliance on this document by any other person who did not receive this document directly from Baillie Gifford.