The COVID-19 pandemic has reinforced the strength of the innovative life sciences sector

The COVID-19 pandemic propelled life sciences to the forefront of global events. It has demonstrated the rapid pace of innovation possible. Strong fundamentals in the UK support the continued growth of the life sciences sector.

Continued strong growth in global clinical trial pipeline:

3%

Continued strength of UK life sciences industry

4/10 **£81**bn

18%

12%



50%

Innovation in clinical trials accelerated

The COVID-19 pandemic has required rapid acceleration in the processes of discovery, development and regulatory approval to enable innovation to the Medicines and Healthcare products Regulatory Agency (MHRA), is a sophisticated regulator and has overseen the safe and rapid approval of vaccines. Drug development timelines have been condensed and adaptive trial designs have enabled modifications in studies after they have begun.

Regulators such as the Food and Drug Administration (FDA) and European Medicines

Agency (EMA) have issued guidance in support of virtual trials, with this flexible approach following on from a pre-existing trend acknowledging the reach patients. The UK regulator, benefits of real-world evidence (RWE) in clinical development⁸. The logistical and clinical benefits of either fully or partially virtual trials are varied; participants can be drawn from a wider geographic area which does not need to be dictated by the location of infrastructure, costs can be more carefully managed, whilst patients can react to treatments in an environment which more closely resembles their normal lives.

An agile approach to clinical trials

The COVID RECOVERY trial, launched in March 2020 and led by the University of Oxford, is the world's largest trial of potential COVID-19 treatments. The study has been able to scale at speed and quickly rotate through unviable treatment options due to the close collaboration between the broad range of organisations involved, as well as a focus on simplification to minimise the barriers to patient inclusion, and innovative use of the NHS DigiTrials platform to log patient data.



ed for continued growth post-CO

Health expenditure

as a share of GDP.

projection – 2030⁹

20%

16-12%

12-14%

8-10%

8%

The life sciences sector has attracted increased capital flows over the last three years, with the value creation opportunity presented by global macro trends encouraging investment in the sector.

With data suggesting that healthcare spending will continue to increase beyond the rate of economic growth, the global life sciences sector is well positioned to be an attractive proposition for investment over the medium term.

£2.8bn

81m

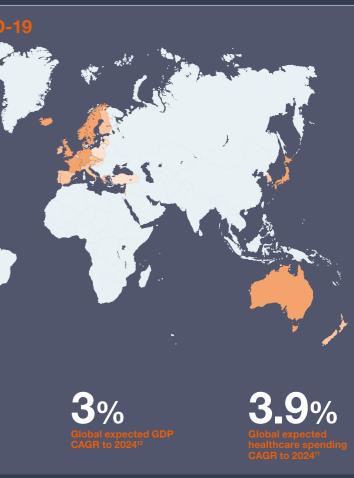
Messenger RNA (mRNA) is a technology at the forefront of the Third Wave of medicine (see next page).

Whilst mRNA research has been ongoing for decades, it is the approval of mRNA COVID-19 vaccines in 2020 which has seen this type of therapy propelled into the public consciousness.

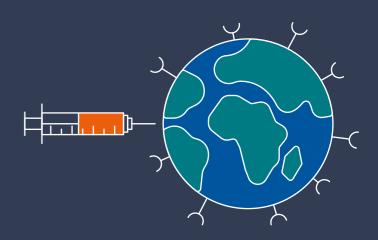
When introduced to the body, mRNA vaccines induce the creation of proteins, enabling the body to launch an immune response against a given virus. Two of the more prominent approved COVID-19 vaccines, from Pfizer and Moderna, use this technology, with both illustrating high levels of efficacy. As well as the increasing body of evidence which shows positive outcomes for patients, mRNA treatments also have significant benefits from a manufacturing perspective. The cell-free process of production reduces complexity significantly, whilst clinical batches can be generated within a very short period of a sequence encoding the immunogen being available. Research is ongoing into mRNA treatments for HIV, sickle cell disease and cancer, with the funding and research in this area which has been accelerated by COVID-19 likely to lead to further breakthroughs in treatments for other illnesses.

Please refer to page 140 for references

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mRNA vaccines further proof of coming-of-age status of Third Wave technologies



Established leadership in Third Wave therapies

"First Wave"

1950s

 $\left(\right)^{2}$

"Second Wave" Small molecule drugs, market 1990s dominated by large pharmaceutical companies.

Large molecule (antibody therapies and enzyme replacement therapies).

02

The "Third Wave" Today

> Advanced biologics and genetic medicines such as gene therapy and cell therapy and DNA/RNA medicines.

ibn

c.1000

20

9 out of 11

20%

ппп

UUU

What is cell and gene therapy?

Cell therapy

Cells cultivated or modified outside the body before being injected into the patient. Cells may originate from the patient (autologous) or a donor (allogeneic).

Cells re-engineered utilising ex vivo gene therapy to, for example, target and kill cancer cells

6 T-cells extracted from a patient's blood*

*Autologous Cell therapy

Ex vivo gene therapy Uses host cells engineered to express a gene of interest which are then transplanted into the body.

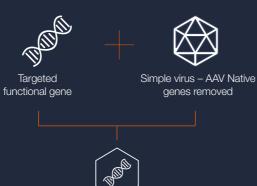
Genetically altered

T-cells infused

back into patient through an IV

In vivo gene therapy

The insertion of genes via a carrier, like an AAV virus, into target tissue to replace a mutated gene which has caused a disease.



Please refer to page 140 for references



The "Third Wave" of healthcare has seen the arrival of advanced therapies harnessing the power of genetics and the patient themselves to treat disease. With its potential to address areas of high unmet medical need and transform outcomes for patients, our belief is there is still significant opportunity ahead in the Third Wave and it has the potential to power healthcare innovation for decades to come.





Syncona's model is complementary to Third Wave companies

Platforms have strategic value in cell and gene therapy. Engineered cells and viruses are highly technical and require complex manufacturing and supply chains. Third Wave products once approved can be delivered by a single integrated platform, presenting a highly modular, scalable opportunity for those with platform expertise.



Please refer to page 140 for references

Gene Encapsulated in AAV





Market context