Breakthrough Medicines 2021

Dec. 7, 2021 by Chris De Savi

It has been a momentous year of innovation for drug discovery while the world is still facing the deadliest pandemic in its history. Below is highlighted some of the most impactful breakthrough medicines. These approved or emerging treatments will have a profound positive and meaningful impact on human lives.

Some of these medicines such as the COVID-19 vaccines and new oral anti-virals have been discovered at an unprecedented pace. The vaccines from both Pfizer BioNTech SE and Moderna also represent novel technologies to treat disease using messenger RNA (mRNA) to teach our cells how to make a protein that will trigger an immune response inside our bodies. A gargantuan global effort. Amazing innovation.

A new treatment for Ebola virus and the first vaccine to treat malaria passed us by.

Inmazeb is a combination of three antibodies that target a protein on the surface of the Ebola virus. In clinical trials, 66% of the 154 people who received Inmazeb survived, compared to only 49% of the 153 people who didn't. Profound.

There are over 219 million cases of malaria globally and half a million result in death. Mosquirix generates an immune response against Plasmodium falciparum, which is among the deadliest of the five parasites that cause malaria, and the most prevalent strain throughout Africa. The inoculation is about 50% effective against severe malaria but remains one of the best ways to prevent this deadly disease.

Children diagnosed with Hutchinson-Gilford progeria syndrome (HGPS) rarely live beyond 15 years of age. Lonafarnib, a farnesyltransferase inhibitor, is an oral medication that helps prevent the buildup of defective progerin or progerin-like protein. The effectiveness of this drug was demonstrated in 62 patients from 2 single-arm trials that were compared to matched, untreated patients from a separate natural history study. Compared to untreated patients, the lifespan of HGPS patients treated with drug increased by an average of 3 months through the first 3 years of treatment and by an average of 2.5 years through the maximum follow-up time of 11 years. The new drug also reduces symptoms of heart and bone problems associated with the rare condition, which affects ~400 children worldwide.

NTLA-2001 could potentially be the first curative treatment for ATTR amyloidosis. It is the first

investigational CRISPR therapy candidate to be administered systemically, or IV, to edit genes inside the human body. Robust preclinical data, showing deep and long-lasting transthyretin (TTR) reduction following in vivo inactivation of the target gene, supports its potential as a mono-therapy. Interim PhI clinical data confirms substantial, dose-dependent reduction of TTR protein following a single dose of NTLA-2001. It is very early days but if this drug if approved it could be ground-breaking.

Amazing innovation prospers #pharmaceuticals #healthcare

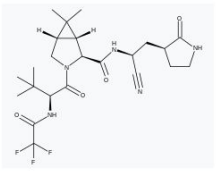
2021 Breakthrough Medicines

COVID-19 Vaccines and Oral Anti-virals

Pfizer-BioNTech (Comirnaty)
Moderna (Spikevax)
Mechanism mRNA vaccine

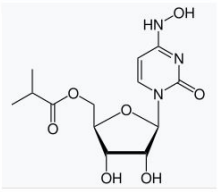
Oxford-AZ (Covishield)
Janssen
Sputnik V
Mechanism Viral vector vaccine

Sinovac (Coronavac)
Covaxin
Mechanism Inactivated virus



PF-07321332 (Paxlovid)
To treat COVID-19
RoA PO
Mechanism 3C-like protease inhibitor
Mw 499.5; clogP 2.1; tPSA 131.4
(in combination with ritonavir)

Pfizer



Molnupiravir (Lagevrio)
To treat COVID-19
RoA PO
Mechanism RNA-dependent RNA polymerase induction of lethal mutagenesis
Mw 329.0; clogP -1.5; tPSA 140.9

MERCK
INVENTING FOR LIFE

New treatment for Ebola virus

Atoltivimab/maftivimab/odesivimab (REGN-EB3; Inmazeb)
To treat Ebola virus
RoA IV
Mechanism Human mAbs that target glycoprotein surface of Ebola virus

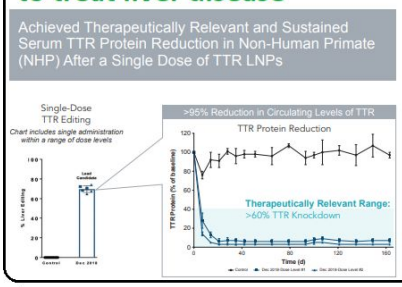
REGENERON
SCIENCE TO MEDICINE

The first vaccine for malaria

RTS,S/AS01 (Mosquirix)
To treat malaria
RoA IM
Mechanism Recombinant protein-based malaria vaccine

gsk GlaxoSmithKline

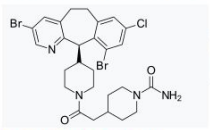
First investigational CRISPR therapy candidate to treat liver disease



NTLA-2001 (Investigational)
To treat transthyretin amyloidosis (ATTR)
RoA IV
Mechanism CRISPR/Cas9 genome editing targeting human TTR gene

Intellia
THERAPEUTICS

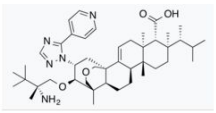
New rare disease treatment to prevent premature aging



EIGER BIOPHARMACEUTICALS

Lonafarnib (Zokinvy)
To reduce death due to Hutchinson-Gilford progeria syndrome
RoA PO
Mechanism Farnesyltransferase inhibitor (FTI)
Mw 638.8; clogP 5.3; tPSA 79.5

Novel anti-fungal to treat yeast infections



Ibrexafungerp (Brexafemme)
To treat vulvovaginal yeast infection
RoA PO
Mechanism Triterpenoid antifungal
Mw 730.0; clogP 3.3; tPSA 125.4

SCYNEXIS

First complement protein C3 inhibitor for treatment of rare blood disease

Pegcetacoplan (Empaveli)
To treat paroxysmal nocturnal hemoglobinuria (PNH)
RoA s.c.
Mechanism Complement protein C3 inhibitor

Apellis

Novel treatment for Alzheimer's disease

Aducanumab (Aduhelm)
To treat Alzheimer's disease
RoA IV
Mechanism Amyloid-beta directed mAb that targets amyloid beta
Mw 145912.3

Biogen

2021 Breakthrough Medicines