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The Top Five (Non-COVID) Pharma Stories Of 2020

by Kevin Grogan

The (virtual) doors closing on the J.P. Morgan Healthcare Conference is the sign for the biopharma industry that the new year has truly started, but before we get too far into 2021, *Scrip* has taken a look at five of the biggest non-COVID-19 story themes of 2020 in no particular order.

No M&A Mania But Major Deals Still

Unsurprisingly, M&A activity in 2020 was less frantic than pre-pandemic days and the year was not dominated by mega-mergers such as in 2019 when *Bristol Myers Squibb Company* bought *Celgene Corporation* and *AbbVie Inc.* acquired *Allergan plc*. Neverthless, and despite discussions being limited to Zoom and Team meetings, there were plenty of deals struck as business development carried on apace.

For much of the year, it looked as though *Gilead Sciences, Inc.*'s $21bn purchase of *Immunomedics, Inc.* and its triple-negative breast cancer antibody-drug conjugate Trodelvy (sacituzumab govitecan) would be the biggest transaction of 2020, coming six months after Gilead paid $5bn for *Forty Seven Inc.*. However, December came and *AstraZeneca PLC* agreed to splash out $39bn on *Alexion Pharmaceuticals Inc.*, leaping to the top of the rare disease tree by getting hold of the blockbuster Soliris (eculizumab), its follow-up Ultomiris (ravulizumab), and a pipeline of 11 molecules.

A big deal but even that acquisition would have been dwarfed if the rumor mill had got it right earlier in the year that AstraZeneca had approached Gilead about a potential merger that could have created a healthcare giant worth over $230bn at the time. Nothing came of that but the AstraZeneca/Alexion deal could pave the way for a return of big M&A in pharma.

(Also see "*Gilead Buys Pipeline-In-A-Product With $21bn Immunomedics Deal*" - Scrip, 13 Sep, 2020.)

(*(Also see "AZ's Alexion Acquisition Looks Astute Bit Of Business*" - Scrip, 13 Dec, 2020.))

(Also see "*AstraZeneca Gilead Merger Intriguing But Unlikely*" - Scrip, 8 Jun, 2020.)

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Lots Of Eyes On Inclisiran…

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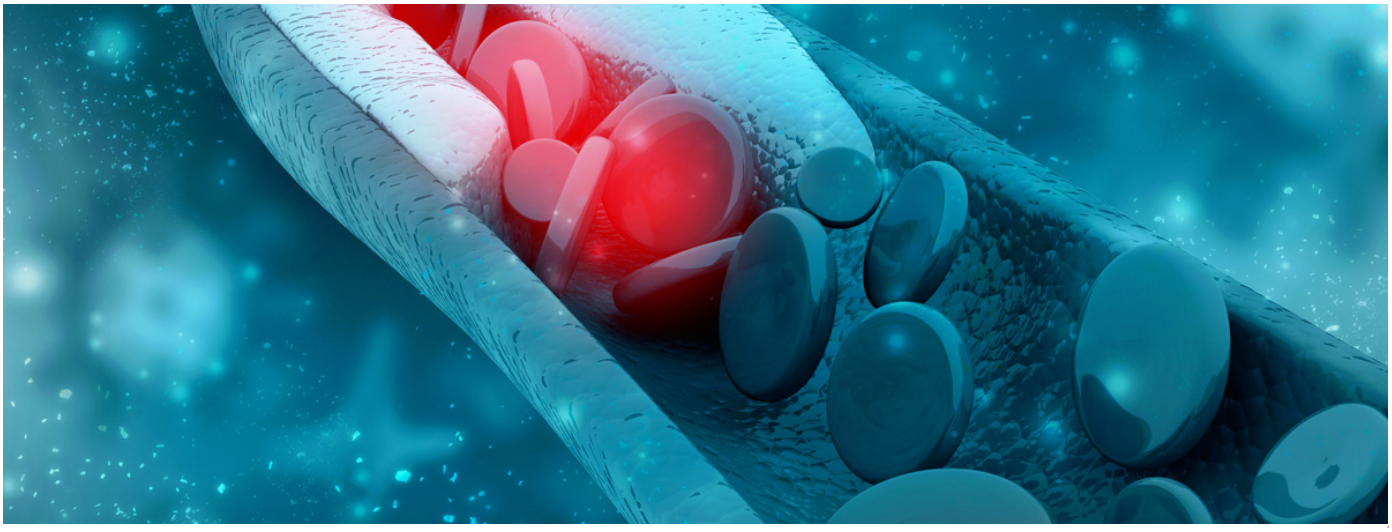

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On 6 January in 2020, *Novartis AG* completed its acquisition of *The Medicines Company*, getting hold of inclisiran, a cholesterol-lowering medicine that was rarely out of the spotlight last year. The first-in-class small interfering RNA treatment targeting PCSK9 could be a gamechanger in the cardiovascular and is expected to succeed commercially where other PCSK9 inhibitors have failed partly because of its twice-yearly administration.

Inclisiran was approved in Europe on 11 December as Leqvio and Novartis CEO Vas Narasimhan believes it will be a blockbuster. The drug is the subject of a pioneering UK agreement, which combines early access to patients with atherosclerotic cardiovascular disease with support from the National Health Service to get patients on the medicine. That pact includes a trial evaluating the use of the drug to patients at very high risk of having their first cardiac event and the creation of a consortium to look at manufacturing synergies to improve how the UK makes oligonucleotide medicines such as inclisiran.

Narasimhan told *Scrip* that "it's quite exciting to get that going," but the excitement around inclisiran did not end there as the year ended with a less welcome event when the US Food and Drug Administration issued a complete response letter due to "unresolved facility inspection-related conditions" at a third-party site in Italy.

(Also see "*Novartis CEO: Inclisiran Could Be Largest Medicine In NHS History*" - Scrip, 30 Jan, 2020.)

(Also see "*EU First To Approve Novartis's Cholesterol Drug Leqvio*" - Scrip, 11 Dec, 2020.)

(Also see "*Novartis' Inclisiran Grounded In The US By FDA Inspection Restrictions*" - Scrip, 21 Dec, 2020.)

…But More Eyes On Aducanumab

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Clearly COVID-19 dominated the pharma world last year but 2020 saw frenzied debate about whether the industry had finally developed the first disease-modifying drug for Alzheimer's disease in the shape of *Biogen, Inc.* and *Eisai Co., Ltd.*'s amyloid-targeting therapy aducanumab.

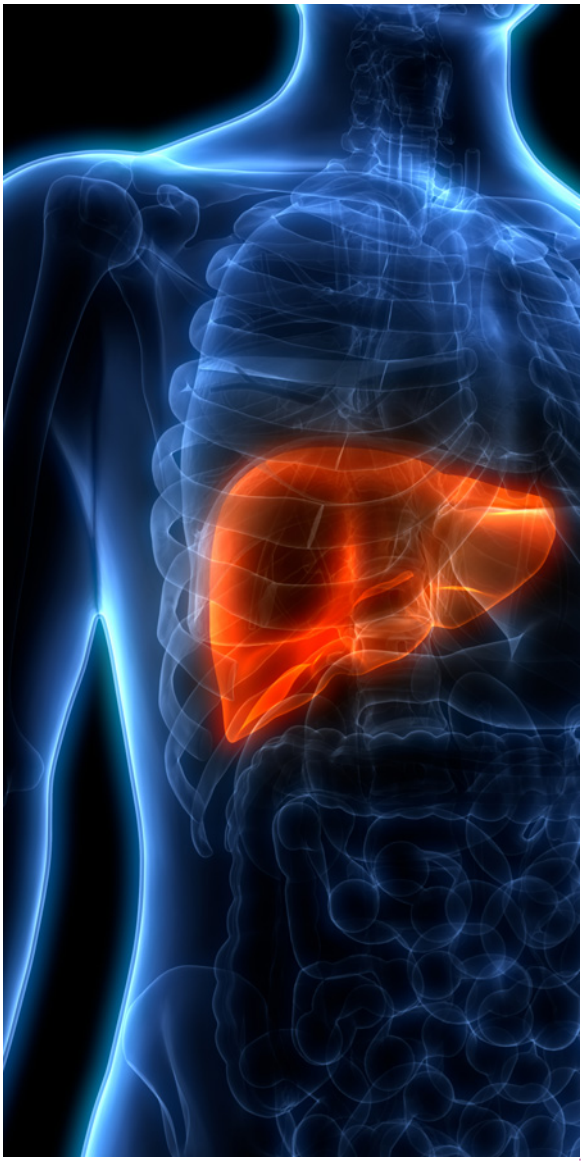
The aducanumab saga rattled on through the whole of the year following Biogen's surprise announcement late 2019 that despite the failure of two Phase III trials, a futility analysis was incorrect and that one of the studies, the EMERGE trial, was actually successful with the highest dose of aducanumab. The validity of that claim was dissected throughout 2000 up to an eagerly anticipated FDA advisory committee on 6 November and briefing documents ahead of the event suggested that the agency's leadership were very gung-ho about aducanumab.

However, the advisory committee panel voted not to recommend approving the drug and was skeptical about the briefing package in which the FDA revealed it worked closely with Biogen on the regulatory filing. All very controversial and while the panel rejected aducanumab, it does not necessarily mean that the FDA will. Analysts keep changing their minds on whether we will see approval for the first novel drug for any Alzheimer's indication since 2003 and a decision is expected by 7 March.

(Also see "*Aducanumab Panel Pushes Back On Rosy FDA Outlook, Could Derail A Swift Approval*" - Scrip, 7 Nov, 2020.)

(Also see "*Outlook For Biogen's Aducanumab Brightens Based On FDA Clues*" - Scrip, 4 Nov, 2020.)

(Also see "*Biogen's Twisting Path To The Aducanumab Advisory Committee*" - Pink Sheet, 4 Nov, 2020.)



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Nothing For NASH Yet

2020 was supposed to be the year when the first drug would finally be approved for non-alcoholic steatohepatitis (NASH), the common but serious liver disorder which has the potential to become a large and lucrative market. However, last year was littered with late-stage failures.

Intercept Pharmaceuticals, Inc.'s FXR agonist obeticholic acid (OCA), already approved for the smaller liver indication primary biliary cholangitis under the brand name Ocaliva, was leading the field until it got an unexpected complete response letter from the FDA at the end of June. Intercept accused the FDA of moving the goalposts during its review but the agency insisted it required further data from the ongoing REGENERATE study.

That surprise came just weeks after *Genfit SA* reported that its PPAR alpha/delta agonist elafibranor failed a Phase III trial that was expected to put it second-in-line to market. These events have blown the race to market wide open and the likes of *Galmed Pharmaceuticals Ltd.*'s' synthetic fatty acid/bile acid conjugate Aramchol, AbbVie's CCR2/5 antagonist cenicriviroc or *Madrigal Pharmaceuticals, Inc.*'s THR beta agonist resmetirom, all currently in Phase III, could still be first for a NASH approval. Gilead, with its FXR agonist cilofexor and ACC inhibitor firsocostat, plus *Inventiva S.A.*. and *89bio, Inc.* are companies that also have their eye on the prize.

(Also see "*Year Of NASH Upheaval Means Incremental Data At AASLD*" - Scrip, 18 Nov, 2020.)

(Also see "*Intercept's CRL Just Continues Upheaval In NASH*" - Scrip, 29 Jun, 2020.)

(Also see "*RESOLVE-IT Failure Dashes Genfit's NASH Hopes*" - Scrip, 12 May, 2020.)

Brexit Done But Not Dusted

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2020 saw the UK endure the double-whammy of COVID-19 and Brexit and the pharma industry spent much of the year urging the government to finally sign a deal with the EU before the end of the transition period on 1 January. On Christmas Eve, pen was finally put to paper four and a half years after Britain decided to leave the union, a move welcomed by the Association of the British Pharmaceutical Industry and the European Federation of Pharmaceutical Industries and Associations, who declared a deal was in the best interest of patients in the UK and the EU and meant ongoing collaboration in scientific research and medicines safety.

There are many questions that remain unanswered, not least the flow of medicines and delays at borders due to new customs and border checks. However, the UK government has been looking to promote the country as one that will continue to be a hub for the life sciences now that Brexit is a reality.

Key to that will be the role of the UK's Medicines and Healthcare products Regulatory Agency, which is now a fully freestanding national agency following the end of the Brexit transition period. When still under the auspices of the European Medicines Agency, the MHRA was the first regulator worldwide to issue emergency authorizations to *Pfizer Inc.*/*BioNTech SE*'s mRNA-based

COVID vaccine and COVID-19 Vaccine AstraZeneca, but now on its own it has recently launched the Innovative Licensing and Access Pathway (ILAP) intended to reduce the time taken to get new drugs to market.

(Also see "*Pharma Breathes Sigh Of Relief Over Brexit Deal*" - Scrip, 24 Dec, 2020.)

(Also see "*UK Authorizes 'Gamechanger' AstraZeneca Vaccine Despite Data Doubts*" - Scrip, 30 Dec, 2020.)

(Also see "*UK Vaunts 'New Era' In Drug Approvals*" - Pink Sheet, 4 Jan, 2021.)