

Healthcare investment case strengthened by FDA

When news of Republican Scott Gottlieb's potential nomination as new chief of FDA came out in early March, there was a collective sigh of relief from both FDA staff and from key democrats who follow FDA. This general level of comfort with his nomination stemmed from the fact that Gottlieb had already worked in several federal government roles including as Deputy Commissioner for Medical and Scientific Affairs at the FDA from 2005 to 2007. Indeed, he is credited with work to initiate the early development of the FDA's generic drug user fee program among other directives. He was therefore known to and trusted by the FDA staff. He was also a member of the White House Biodefense Interagency working group and well known to Republicans and Democrats. In short, he was a far more popular and less divisive choice than other candidates which had been mentioned.



Scott Gottlieb

He got off to an excellent start with the confirmation hearings - doing a great job of communicating with his critics to allay any fears they may have had regarding conflict of interests etc.. He was confirmed by the Senate on May 9th, 2017.

In the 4 or 5 months since his appointment, he has proven to be a master communicator. In these uncertain times, he has positioned FDA as an island of tranquillity in an otherwise turbulent world. He has managed to shield the FDA from Trump critics by addressing key politically sensitive issues head on.

His stance on the opioid epidemic is a good example of how politically astute Gottlieb appears to be. Elected officials are coming under increasing pressure to do something about this issue. This is a political hot potato. In July, after just 2 months in office, Gottlieb issued a statement in which he said; *“Reducing the scope of the epidemic of opioid addiction is my highest immediate priority as Commissioner”*.

He has requested a ‘fresh look’ at the key features and where FDA can have an impact. FDA has already requested the removal of an opioid product (Opana from Endo Pharmaceuticals) as its misuse is now considered to outweigh its benefits. The opioid crisis is considered by many to be an unsolvable problem. However, Gottlieb did well to get out ahead of any potential critics on the topic. We can expect that the approval of any opioid based products will come under increased scrutiny – even those with abuse deterrent properties and the REMS programs will become more onerous.

On August 18th, Gottlieb scored another win when President Trump signed into law an Act which includes the reauthorisation of the so called “PDUFA” through to September 2022. This act authorizes FDA to collect fees from companies that produce drug and biologics. It was created in 1992 and it outlines just how much money the industry will pay to fund the reviews of new drugs and medical devices. Without it, the FDA would have to seriously curtail its activities, as a result of which the overall drug approval process would have become much slower. Since it is precisely this process that forms an integral part of most of the biotech investment cases – from the smallest to the largest companies – it is one of the key metrics that influences the short and longer term potential of the sector, hence its importance. This reauthorisation secures funding for the next 5 years. This act passed by almost unanimous votes in both the House and Senate – which is very unusual.

At this point Gottlieb appears to have everyone behind him and he is sitting at the head of a very well-resourced, well-financed FDA, where staff morale is very high by all accounts.

As a result, the drug approval climate has been described by specialists and investors as fantastic. Is it correct to credit Gottlieb with this? In truth, the climate for drug approval has been good for some time now but the tone and belief in FDA has changed. Gottlieb has certainly played a clear roll in raising the awareness of how great it is. Other members of the leadership team within the FDA such as Janet Woodcock, who is heading the Center for Drug Evaluation and Research and Rick Pazdur, who is heading the FDA's Oncology Center of Excellence, have been with the FDA for many years and are working hard to instigate new approaches to modernize the FDA. Perhaps perception is only now catching up with reality.

In recent speeches, Gottlieb has been outlining plans for the FDA to adopt new and more modern methods to collect the clinical information used to make regulatory decisions. These plans include greater flexibility in clinical trial design to allow enhanced enrichment for patient characteristics that correlate with benefits or reduce side effects. This will go hand in hand with the use of combined-phase studies. Rather than the classic three sequential series of clinical studies – FDA is moving towards one adaptive or seamless trial where the phases are separated by interim looks rather than having to complete one trial before initiating the next. According to Gottlieb: *“By using one large, continuous trial, it saves time and reduces costs”*. To improve how FDA evaluates the data from these and more classic clinical trials – Gottlieb is directing an effort to try and increase the agencies investment in new computing tools and algorithms. These tools will facilitate better modelling and simulation.

To drug developers of course this all sounds great and it should facilitate faster more efficient drug development and reduce time to market.

What is perhaps even more exciting to innovative healthcare companies is Gottlieb’s stated commitment to reduce the cost of drug development. We believe this to be the first time in history being a specific focus of the FDA and the perception at least has been that it was not of concern to the agency in the past. So what has changed?



We do not want to suggest that these initiatives are politically motivated but the tone does play into the whole discussion of drug pricing. Another political hot potato as we have described at length in our piece on drug pricing last year! There have been calls for

the FDA to do more to reduce drug pricing. The FDA evaluates the safety and efficacy of a drug candidate in light of its proposed indication – they do not have the power to decide the ultimate price of that drug. Gottlieb however, correctly asserts that the cost of drug development is directly linked to the commercial cost of medicines. In a speech in September 2017 Gottlieb stated: *“We are on an unsustainable path, where the cost of drug development is growing enormously, as well as the costs of the new medicines”*. His answer is to improve the efficiency of the drug approval process. He and his team are doing this by initiating these new initiatives but also by giving more clear guidance to drug development companies to improve communication with all stake-holders of the industry - such that the drug development process is de-risked.

Gottlieb knows how Wall Street works. He aims to reduce the risk and uncertainty that makes drug development so costly. As he knows full well, lower risk also lowers the cost of capital. In effect his plan is simple – reduce the cost of drug development to encourage investors to pour more capital into healthcare companies to fuel further innovation and medical breakthroughs.

This is the ultimate win: win. Wall Street wins through increased return on investment relative to risk. Innovative healthcare companies would see an improvement in funding and patients benefit from additional new and improved treatments reaching the market. Ultimately, this should all contribute to reducing the overall cost of healthcare.

The modernisation process at FDA has been underway for many years. Indeed perception does seem to be catching up with the reality that this is a ‘fantastic’ environment for drug development. Scott Gottlieb role is still critical – he is bringing a clear understanding of the issues from the perspective of the different stakeholders; he is excellent at communicating the FDA message and perhaps most importantly under his leadership – there is considerable enthusiasm that it can be done.