POSITION PAPER
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INNOVATIVE MEDICINES NEED INNOVATIVE (NOVEL) EXCIPIENTS

How Novel Excipients can foster patient access to innovative and affordable medicines

What is the problem?

There is no regulatory pathway for the independent approval of **novel excipients** in Europe, which is a strong hindrance to innovation in the development of medicines. This is highlighted by delays in the approval of COVID-19 vaccines in the EU.

For example, synthetic lipids, classed as novel excipients, are used in the development of mRNA COVID-19 vaccines. Each Marketing Authorisation Application (MAA) for COVID-19 vaccines using these materials triggered the repeated review of the lipid Chemistry Manufacturing and Control (CMC) information. These vaccines did not enter the EU market first and one reason to be considered is the missing independent review for novel excipients.

The importance of excipients and novel excipients:

Excipients bring specific and essential functionalities to medicines. Described as substances other than the active ingredient (API) which have been appropriately evaluated for safety, they are intentionally included in a drug delivery system. A novel excipient is one used for the first time in a medicine, at an unprecedented use level or new route of administration. Their use can foster innovation and help to formulate drugs improving their efficacy, quality and safety.

It is estimated that as many as 15% of drug candidates fail during Phase I clinical studies because of insufficient solubility, resulting in low bioavailability.

Regulatory innovation will trigger more extensive use of novel excipients.

The absence of an independent regulatory pathway delays time-to-market and innovator companies may choose not to use novel excipients to formulate active substances (APIs). Today, novel excipients are only approved together with the API for a specific medicine. This requires multiple submissions of large data sets of confidential information and repeated assessments by regulators which is **inefficient and prolongs approvals**.

Why is action needed now?

Advanced treatments and therapeutics, particularly in the area of biologics, for example m-RNA vaccines, increase the need to use novel excipients. Avoiding their use is no longer a viable strategy and Europe cannot fall behind if the future needs of EU patients are to be met without delay.

The lack of a workable system for novel excipients in the EU has a negative impact on Europe's innovative medicines development compared with other countries such as China and USA, to the detriment of EU patients.

If an independent regulatory pathway existed to protect the IP of novel excipients, it would encourage collaboration between their discoverers and SMEs to develop innovative medicines.

For the COVID-19 vaccine examples, lipid manufacturers could have submitted CMC information directly to the EMA enabling efficient review of the data required to support several MAAs, protecting the excipient manufacturers' IP at the same time.

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Adapting the EU framework to develop high-quality, effective and safe medicines:

- A modular approach such as the Master File System for APIs (ASMF) should be extended to novel excipients providing an independent review. Data needs for novel excipients are the same as for APIs and regulatory equality is essential.
- Cross-referencing a novel excipient Master File in a MAA would ensure a full review by EMA while shortening approval times and simplifying authorisation procedures.
- Novel excipient manufacturers could work in parallel with developers of medicines and reduce approval times, especially in crisis situations.
- The IP of the novel excipient will be protected.

These initiatives would be complementary to the European Commission's Patient-centred Pharmaceutical Strategy, encouraging the development of Advanced treatments and therapeutics and increasing EU citizens' access to high-quality, effective, and safe medicines.
Barriers to innovation would be reduced making Europe more competitive, agile and, on a level playing field with other global markets (such as China and USA).

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