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Stepwise market approval of new pharmaceuticals: Principle of hope should not displace principle of safety

Berlin, 04.04.2016: There is an on going debate at European level to noticeably shorten the approval process of new pharmaceuticals by the way of lowering the approval standards for drug manufacturers. Especially patients with severe diseases and lack of therapy options are said to benefit from an earlier access to new drugs, argue supporters of these plans. From the perspective of the statutory health insurance system such a approach should only be implemented with the utmost caution. “However comprehensible the hope for healing or alleviation of an illness is, it should not be achieved by partly renouncing the principles of safety as a precondition for the market approval”, states Johann-Magnus v. Stackelberg, deputy chairman of the Statutory Health Insurance Funds.

From the perspective of the Statutory Health Insurance Funds a solid, scientific evidence-based foundation for the examination of efficacy and risk of new drugs prior to approval remains the top priority. "We cannot fall behind safety standards the legislator set in the 1970s following the painful experience with the Contergan scandal. Robust, comparative phase III trials are essential to confirm the alleged effectiveness of a drug and to identify possible severe side-effects during a test phase, in which voluntary study participants are intensely monitored and medically cared for, before the product is made available to the general public. Accelerated approvals of drugs therefore must strictly remain in those cases of real medical need. Only in these cases fast track market approvals can be justified, as otherwise they may result in misjudgments of the efficacy, risks and side effects of a drug due to the insufficient database at the time of market approval”, says v. Stackelberg.

Current experience: industry hardly provides additional data

Already today, the European Medicines Agency (EMA) has several options to accelerate the access to new drugs by patients as part of the approval process (i. e. the conditional authorization or authorization under exceptional circumstances). In spring 2014, the EMA has started a pilot project (“adaptive pathways”) to merge the existing special pathways under a unified concept for an accelerated

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market access for drugs, despite limited database. The new drug would then either be initially approved only for a small group of patients with unmet medical needs. Or a conditional approval would be granted based on uncertain data. In these specific cases the EMA would lower the conventional evidence standards for approval. Amongst other, specifically the highly relevant phase III studies, i. e. comparative tests with lager groups of patients, which are crucial in assessing the safety issues are no longer mandatory. In return, drug companies commit to submit additional studies after the approval relating to the safety and effectiveness of a drug. If after a certain period of time sufficient evidence was available, EMA would upgrade the initially conditional to a “full approval” or extend the initially limited application area step by step to new ones.

However, current experience with the already possible accelerated approval process already show, that study results promised by manufacturers are not submitted at all, submitted delayed in time or with quality-lowering deviations from the original terms of approval. Between January 2006 and June 2015, the EMA approved a total of 490 drugs, of which 26 received a conditional approval, as part of the existing fast track process. Of these 26 drugs only 10 were upgraded to a regular approval on the basis of data provided by manufacturers in subsequent phases (38.5%). On average, it took up to 5 years for the upgrade to full approval, years in which the effectiveness and safety of the drug were not ensured at regular standards. It can be observed that the EMA lowered its original pre-approval requirements following the market release of a drug. Also, in no case has the approval been withdrawn because of not adhering to the approval conditions and it is unknown whether other sanctions were imposed.

Late delivery of studies must be sanctioned

"We need to ensure that the pharmaceutical companies really deliver those promised studies after approval and this at an evidence standard and quality, as the right of the patients to a safe and effective treatment necessitates", cautions v. Stackelberg. "If the approval authorities do not effectively enforce the obligation of data submission, then the national social systems must do it. “ Therefore, the Statutory Health Insurance Fund proposes an adjusted reimbursement on the basis of mandatory benefit assessments, which have to be repeated on a recurrent basis. If the companies do not present the requested studies after expiry of the submission deadline, the reimbursement should be restricted in an adequate manner.

Given the already well-advanced plans of the EU, v. Stackelberg generally lowers the expectations regarding the reimbursement of such conditionally approved drugs. The less data is available at approval stage, the thinner is the basis for an initial judgement and hence for a vote on additional benefits by the Federal Joint Assembly. V. Stackelberg: "A stepwise approval can only translate into a level of reimbursement that mirrors stepwise the respective level of knowledge at a certain point in time and takes into account the initially higher uncertainty of the additional benefit."

Drawing right conclusions from a stepwise approval

If the stepwise approval of new drugs will prevail, also the doctor-patient-conversation needs to adapt. "Patients as well as physicians have the right to know what risk of side-effects and interactions they incur. The higher level of uncertainty associated with this type of approval must be communicated to patients and physicians. And much more specific and wider than it is happening today", says v. Stackelberg.