Zusammenfassung der Master-Thesis
von Rossella Belleli

Efficiency indicators for new drugs approved by the FDA from 2003 to 2013

In the last decades, the drug development costs have increased while the number of new drugs has been fairly constant. For this reason, the pharmaceutical industry is under pressure to develop new drugs to treat the numerous yet unmet medical needs in a sustainable way. The purpose of this publication was to investigate if there is evidence of increased efficiency in the development of new drugs (new molecular entities, NMEs and new biological entities, NBEs) approved by the US Food and Drug Administration (FDA) from 2003 to 2013. We analyzed four endpoints (proportion of drugs approved at the first review cycle, number of pivotal trials per drug, average number of patients per pivotal trial, time from submission to approval) which we regarded as efficiency indicators. We analyzed each indicator separately using Bayesian regression models, assessing whether there was any change from 2003 to 2013, while accounting for potential prognostic factors, such as the assignment to a special-designation program.

Two of the efficiency indicators showed improvement (increase in the proportion of drugs approved at the first review cycle and decrease in the number of pivotal trials per drug), one did not change (time from submission to approval) and one worsened (increase in the average number of patients per pivotal trial). The assignment to a special-designation program was always associated with efficiency improvements. We could not identify prognostic factors for the increase in the size of pivotal trials. Therefore, we suggested that new strategies have still to be sought to reduce the number of patients in the pivotal trials and the approval timelines, in particular when developing drugs which are not candidates for special-designation programs.

In conclusion, drugs targeting serious diseases and assigned to special-designation programs had higher chances of early approval, fewer pivotal trials and fewer patients. Cancer drugs have already exploited the use of these programs, while there are still opportunities to increase efficiency for non-cancer drugs, in particular if they target serious conditions.