Drug repositioning has become a matter of intense interest during the past few years. It is an approach to drug development that calls for reinvestigation of candidates that did not succeed in advanced clinical trials (for reasons other than safety) for potential use in other therapeutic indications.

**Discussed in this Report:**

- Intellectual property coverage for new uses of known drugs
- Tool sets for identifying repositioning opportunities and business strategies
- Applicable legal frameworks and regulatory timelines for repositioned drugs
- Case studies of compound repositioning and approaches taken
- Activities of selected key companies in the drug repositioning business
- Financial aspects and economic potential of drug repositioning
Drug repositioning is also known as drug repurposing, reprofiling, or retasking. In “on-target repurposing,” a drug’s known pharmacological mechanism is applied to a different therapeutic indication than that for which it was initially developed. Even more innovative is “off-target repurposing,” which looks for pharmacological mechanisms that have not yet been described for a known molecule. In either case, having previously failed during clinical development is not a criterion for repositioning; the avenue is equally open to drugs that are or have been marketed.

Drug repurposing can have very different commercial implications. These will depend on where the drug comes from, how much accessible data exist, and how well the repurposer can exploit the new value chain created by a successfully repurposed drug. This will to a large extent depend on what sort of intellectual property can be secured for the drug’s new use, as examined in Chapter 2 of Drug Repositioning: Extracting Value from Prior R&D Investments. The repurposer fights an uphill battle against examiners who will scrutinize the prior art for “obviousness,” i.e., any public facts that can be construed to have anticipated the new medical use of a known drug.

Together with expert knowledge in pharmacology, state-of-the-art genomic, proteomic, animal model, and bioinformatics technologies are employed to identify repurposing opportunities and business strategies. These more technology-oriented aspects are discussed in Chapter 3, followed by an outline of the regulatory environment for repurposing in Chapter 4. Here, we discuss the applicable legal framework and show that while repurposing can remove the initial 1–1.5 years of preclinical and Phase I development time (the latter only if no new formulation has to be developed and tested), the later stages of the regulatory review process for repurposed drugs are the same as with new chemical entities. Chapter 5 discusses exemplary cases of drug repositioning and the approaches taken, depending on the intended goal.

### Use Extensions vs. On- and Off-Target Repurposing

<table>
<thead>
<tr>
<th>Therapeutic Field</th>
<th>Molecular or Pathway/Target</th>
<th>Use Extension (Not Repurposing)</th>
<th>Off-Target Repurposing (Rare Cases)</th>
<th>Off-Target Repurposing (High Novelty)</th>
<th>On-Target Repurposing (Medium Novelty)</th>
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Source: H.M. Pharma Consultancy

Drug repurposing has become a new business segment for the life science services industry. Chapter 6 profiles selected key companies that offer platform-based services to identify repurposing opportunities. For the decade ahead to 2020, we predict that cutting-edge repurposing technology will see increasing integration as a standard process of resource utilization, de-risking and acceleration of drug development. In this chapter we also discuss the internal repurposing efforts of Pfizer, Novartis, and Eli Lilly and how these programs tie into their overall development strategies.

Drug Repositioning: Extracting Added Value from Prior R&D Investments concludes with a discussion of the financial aspects, considering the benefits of repurposing for larger, smaller, and startup companies.

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### About the Author

Hermann A.M. Mucke, Ph.D., spent 17 years in academia and industry before he founded H.M. Pharma Consultancy (www.hmpharmacon.com) in 2000 to become an independent pharmaceutical consultant, analyst, and science author. His last industry position was Vice President R&D in a European pharmaceutical company, which he helped to take public on the Frankfurt Stock Exchange in 1999. Since then, Dr. Mucke, who holds a Ph.D. in biochemistry from the University of Vienna (Austria), has become a consultant and advisory board member for several European and American pharmaceutical companies and a regular reviewer of drugs and patents for Thomson Current Drugs and Ashley Publications. Dr. Mucke is based in Vienna.
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