Drug innovation to add societal value

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- The first step to add societal value should is new drugs having been shown to be effective and safe according to accepted international scientific standards.

→ Requirements:

  - Ongoing drug discovery & development
  - Solid and credible (independent) clinical research
  - Sufficient research capacity to do clinical research keeping in step with drug discovery & development
  - Avoid waste: capacity, funding, and investments should be targeted to added value (Chalmers et al., 2014*)*
  - Innovative designs (e.g., intermediate outcomes (long term clinical outcomes), subgroup/interindividual differences, varying Qol appreciations, precision designs (N=few), cumulation of knowledge over time (guided by MA/IPD) et cetera
  - Where possible: research, follow-up and PMS embedded in routine practice

- If a new drug is targeted at a clinical area already covered by other drugs, there should be no problem in accepting the drug for health insurance if:
  - Based on comparative - or even *'competitive'* effectiveness research – real head to head – it is clear that the new drug is as least as effective and safe as already available alternatives.
  - ánd if it is at the same time cheaper; the new drug would obviously also have better cost-effectiveness ratio (cheaper and at least as favourable outcome).

→ Required:

  - International collaboration (research, funding agencies, policy and the private sector) in doing competitive studies.
  - Shared (open) data
  - Address the problems of 'me too' research (e.g., if no reasonable expectation of a drug being at least as good ánd having a lower price, such research is unethical).

- If a new drug has clinically relevant added value as to effectiveness and/or safety (based on competitive effectiveness research) but is more expensive than the already available ones, a transparant policy decision is necessary, balancing added value versus extra cost.

- If the added value is relevant for patients, and if the manufacturer sets a well motivated and affordable price, appropriate policy agreements should in the end be made.

→ Required:

  – Transparent information, negotiation and decision making.
  – International collaboration of purchasers

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- If for legitimate and transparent reasons the expected price is very high, various strategies can be considered, such as:

  - Stimulating corporate societal responsibility (own initiative or regulated). (all profits are in the end paid for by the public). For example:
    - Avoid too high prices in one field (e.g., highly infrequent (orphan) diseases if profits in others (e.g., successful drugs in frequent chronic illness) are good.
    - Longterm cost-effectiveness strategies: setting prices in relation to expected longterm absorption in the market, instead of planning on a shorter term basis.
    - If high prices were accepted by insurers but the market success proves to be more favourable than predicted, prices can be decreased in second instance.

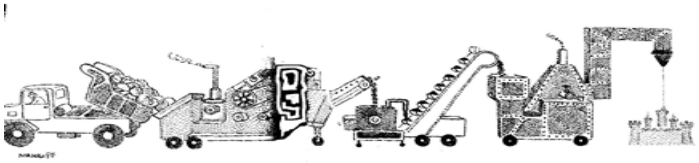
  - Public-private partnerships in societally relevant but economically inattractive markets - e.g., orphan drugs –
    - Public investments can stimulate innovation in manufacturing. Also academic contributions can be integrated.
    - This justifies more governmental (or e.g., EU) influence on price setting or return payment if the drug generates much profit. Society should not pay twice.

- If a drug is completely new (no other drugs available) with added value, and if the manufacturer sets a well motivated and affordable prize, I expect, again, appropriate policy agreements will be made.

- If for legitimate and publicly transparent reasons, the price is very high
  - the same strategies may be considered as proposed in the previous slide. Moreover:
  - In setting indications for use, medical professions and patient organisations to be involved in formulating the criteria, in the context of clinical guideline development (GRADE approach).
  - These activities should better be internationally coordinated as clinical knowledge, clinical implementation and price setting are strongly internationally determined.

- But if discrepancy between need for and availability of essential drugs cannot be overcome (market failure), new policy models might be considered, e.g.,:
  - An independent intermediary organization for drugs provision and taking financial risk, with manufacturers being payed a reasonable, negotiated price.
  - This organisation can be public or be related to collaborating health insurers, so that tax payers or insured can co-decide.
  - Manufacturers (in collaboration with academia) can then focus more on R&D and further innovation.

- We should also consider separating drug discovery & development from clinical (RCT) testing & PMS (Chalmers et al).
- Reasons: independence, efficiency, competitive effectiveness research, transparency.
- This would also better allow public (co)funding of (regulation-relevant) clinical research/PMS.
- Public clinical research fund, with regulated contributions from developers/manufacturers



Some further general considerations

- Developing new business models at the interplay of the public and private interest in drug development and provision is essential.

- Otherwise most innovations in precision medicine (where many drugs will be ‘orphan drugs’) and related population health progress will be missed.

- We should consider costs of effective health care and prevention also as investments in societal and economic gain and progress, since better care and prevention contribute to better and longer societal and occupational participation.

- This emphasizes the importance of expressing the added value of drugs in contributing to functional status and societal participation.

- Societal cost-benefit analysis, in addition to CEA, helps fair assessment as to added value.

The contribution of the health sector to the economy in the EU (Suhrcke, McKee 2005, 2007, 2010)

- One of the most important sectors in developed economies.
- Accounted for 7 % of GDP in EU-15 (5 % accounted for by financial and retail trade sectors).
- Around 9 % of all workers in the EU-25 were employed in the health and social work sector.
- The performance of the health sector affects competitiveness of economy via effect on labour costs, labour market and at macroeconomic level.

- Companies and researchers need to do much better in facilitating evidence-based drug cessation.
- Literature and Cochrane reviews: ’orphan area’ and not popular in industry-related research and funding.
- Avoiding iatrogenic polypharmacy in chronic illness and in multimorbidity deserves high priority.
- It can be considered unethical to present a new drug to the market without an evidence dossier on the possibilities and methods of drug cessation. Regulatory authorities should address this topic.

- For keeping the innovation cycle running effective new drugs should be implemented.
- Preventing really effective innovations from being implemented will – in the end – be impossible as people want progress in fighting disease
- It is wise not to oppose to new, sometimes still costly treatments
- More useful to better deal with both unavoidable and welcome progress, with a vision on longterm improvement.
- What societal innovation would we have ever achieved if our ancestors would not have had a longterm perspective when resources were much more scarce than today.
- In addressing such a long term perspective, both public and private efforts should be involved.