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**DRUG REPURPOSING:
SMART SCIENCE,
DUMB BUSINESS
AND A CLEVER FIX**

Drug Re-purposing

Definition: Use of a drug already approved by FDA for one indication (usually a common disease) for some other indication (often a rare disease).

Two Kinds of re-purposing:

- Off label use

- Secure a new approved indication

Off-label use

- ⦿ Most rare diseases have no treatments
- ⦿ Most RD patients are treated off-label
- ⦿ Off label use is problematic:
 - Weaker scientific rationale
 - Insurance/reimbursement issues
 - Drug can be dropped entirely

Re-purposing via new indications: Smart Science, Dumb Business

◎ Smart science:

- Starting with pharmacologically active substance.
- Starting with a known “safe” drug.
- Leapfrog over pre-clinical studies.

◎ Dumb business:

- Rare disease adds de minimus sales
- Perception/reality of SAE risk to “mother indication” in clinical trials.
- ODA provides little re-purposing incentive

Rare Disease Repurposing Database

- See OOPD Website
- Products with Orphan Designation that are already approved for some other disease.
- Hundreds of potential repurposing opportunities

A Clever Policy Fix

- 30d “Wild Card” Exclusivity (Patent Extension) Vouchers to be issued with approval of orphan repurposed indication.
- Vouchers could be sold. Value to blockbusters going off-patent would be huge. Would provide an incentive for repurposing that is now lacking.

Questions?