

ILSC Conference Series

International Conference on Novel Diagnostics & Affordable Treatments (ICNODAT): Advancing UN Sustainable Development Goal 3 (Good Health & Well-being)

Repurposing of Existing Drugs

29 JUNE 2017

Questions We Will Address

- 1. What are the advantages of developing a drug for a new indication
- 2. What are regulatory hurdles
- 3. What is the best way to regulatory approval





Full Service CRO

Regulatory Affairs
Strategic Planning
Clinical Research
Data Management
Biostatistics
Medical Writing

Clinical Trials Software

eSource for Paperless Trials
Forms Builder
eInformed Consent
ePRO
Encoder
Document Management

Broad Experience Base

Technology Driven

Target Health Experience

Example Drug Expertise

	Target Health CRO Services	Target Health Software Suite
NDA – Pancreatic Insufficiency	Full CRO Services	EDC
NDA – Men’s Health	Toxicology Data Management Monitoring Biostatistics Regulatory Writing	EDC eCTR
NDA – Prostate Cancer	Data Management Biostatistics	EDC
NDA – Head Lice	Toxicology Data Management Monitoring Biostatistics Regulatory Writing	EDC
NDA - Infertility	Data Management Biostatistics	EDC

Example Device Expertise


	Target Health CRO Services	Target Health Software Suite
PMA – Periodontal Disease	Data Management Monitoring Biostatistics Writing	EDC
510(k) – Hair Loss	Data Management Monitoring Biostatistics Regulatory Writing	EDC eCTR
PMA – Adhesion Prevention	Data Management Monitoring Biostatistics Writing	EDC PMA eCopy
PMA – Bone Fractures	Data Management Monitoring Biostatistics Writing	EDC

REPURPOSING OF DRUGS

- Also known as Drug Repositioning, Re-Profiling, Re-Tasking, and Therapeutic Switching
- Original repurposed drugs:
 - Aspirin (ASA) for headache, pain – then prevention of clotting
 - Zidovudine (AZT) for cancer – first antiretroviral for HIV
 - Sildenafil (Viagra®) for angina – erectile dysfunction
 - Thalidomide (Contergan) for morning sickness in pregnancy – now for multiple myeloma and other cancers
 - Gabapentin/pregabalin as an anti-epileptic – now for neuropathic pain and anxiety



Advantages of Repurposed Drug

- Reduced toxicology requirements
 - Drug Substance is the same
 - New indication and new Formulation may provide IP protection
 - Reduced costs to market, depending on the indication
 - More affordability in the market place
- 
- A decorative graphic in the bottom-left corner of the slide. It consists of a network of interconnected nodes and lines, rendered in a light red or pink color. The nodes are represented by small circles, and the lines are thin, creating a complex, web-like structure that suggests a molecular or biological network.

FDA ISSUES

- 505(b)(2) of the Food Drug and Cosmetic Act
- A 505(b)(2) application is one for which one or more of the investigations relied upon by the applicant for approval "were not conducted by or for the applicant and for which the applicant has not obtained a right of reference or use from the person by or for whom the investigations were conducted" (21 U.S.C. 355(b)(2)).



FDA ISSUES

What type of information can an applicant rely on?

- Published literature
- FDA's finding of safety and effectiveness for an approved drug



Working With FDA

- Meet with FDA Early
- Bring Experts with You
- Have a Plan
- If You Do It Right, you will get free consulting from FDA

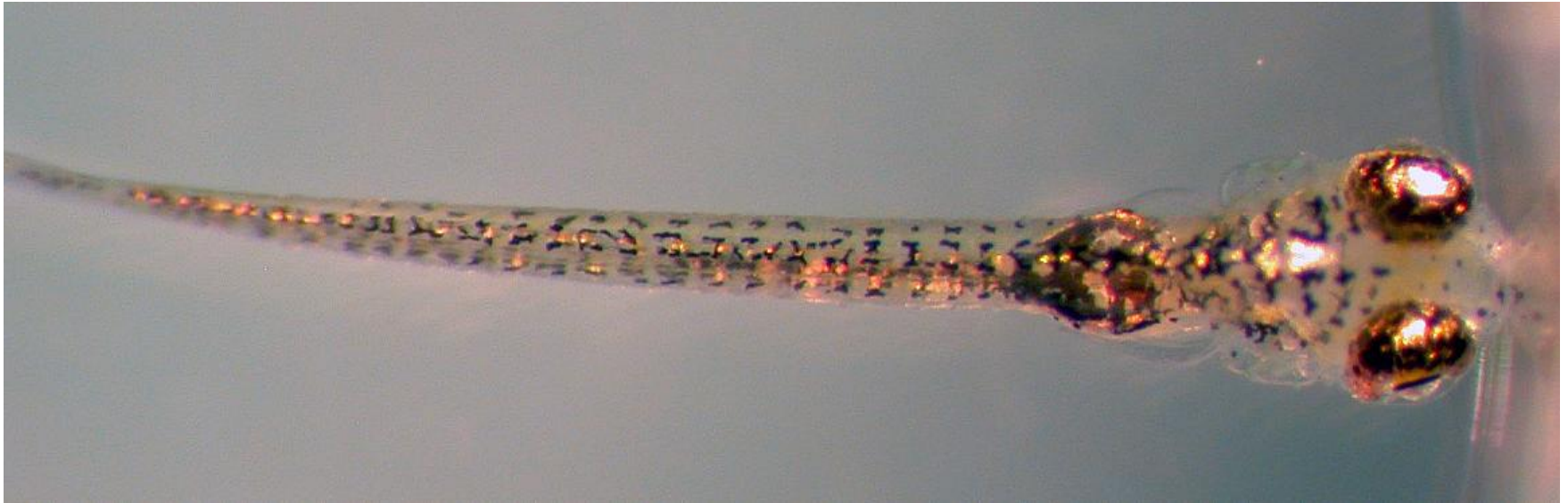


Example for Multiple Sclerosis - Lipitor

- MS is an autoimmune disease in which the body's own immune cells mistakenly recognize cells in the brain and spinal cord as foreign and attack them. This leaves behind scars or lesions and disrupts the ability of nerves to transmit information, resulting in disability.
- In the new study of people with early forms of multiple sclerosis, the statin drug Lipitor cut the risk of developing new brain lesions by about 50% compared with placebo. New lesions are reliable indicators of future MS attacks, according to the study.



Example for Dravet Syndrome - Clemizole



Example for Dravet Syndrome - Clemizole

- Dravet syndrome is commonly caused by a mutation in the Scn1a gene, which encodes for Nav1.1, a specific sodium ion channel found in the brain. Sodium ion channels are critical for communication between brain cells and proper brain functioning.
- UCSF researchers screened 320 compounds and found that clemizole was most effective in inhibiting seizure activity. Clemizole is approved by the U.S. Food and Drug Administration and has a safe toxicology profile.
- “Based on what is currently known about clemizole, we did not predict that it would have antiepileptic effects,” said Dr. Baraban



Thank You



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TARGET HEALTH INC., FOUNDED IN 1993, IS A PRIVATE, NEW YORK CITY-BASED, FULL-SERVICE ECRO, ENGAGED IN ALL ASPECTS OF DRUG AND DEVICE DEVELOPMENT, INCLUDING REGULATORY AFFAIRS STRATEGIC PLANNING, CLINICAL RESEARCH, DATA MANAGEMENT, BIostatISTICS, MEDICAL WRITING AND THE PAPERLESS CLINICAL TRIAL.