Rare Disease Update: Repurposing Drugs Through Computational Biology
October 30, 2018

Exciting therapeutic potential in the repurposing of approved drugs is becoming a reality. Nowhere else does this practice have as much of a potential impact than in the treatment of rare diseases.

Dr. Bloom will discuss the benefits and challenges of drug repurposing, providing historical and contemporary successes with a focus on rare diseases. He will describe how computational biology and precision medicine are being leveraged to expand opportunities, while increasing the speed with which therapies can be brought to patients at a lower cost.

Bruce Bloom, DDS, JD
CEO, Cures Within Reach

Discussant:
Antonio Molina-Pachon, Ph.D.
CEO, GenCo Pharma
Westchester Biotech Project

-a borderless initiative mapping the future for regional and international collaboration

Co-Founders:

Michael Welling, Chair
Partner, Meridian Risk Management

Joanne Gere, Executive Director
Partnership with the Institute for Life Science Entrepreneurship and CUNY

Innovation in Research

Rare Disease Symposium

Tauopathy Colloquium

CAUTION
WATCH YOUR STEP!

Young Investigators

Consortium on Translational Research in the Microbiome

Westchester Biotech Project

EUROPE
Thank You to our Community Partners, Alliance Partners, and Participants!
Rare Disease Update: Repurposing Drugs Through Computational Biology
The Problem

7000+ unsolved rare diseases
500M people affected
Few new therapies each year
New therapy cost/development time
ONE Solution

THE LITTLE ENGINE THAT COULDN'T

A POP-UP BOOK
Retold by WATTY PIPER
A PLATT & MUNK CLASSIC

RAREPURPOSING
CWR Repurposing Definitions

**RAREpurposing**-finding a new rare disease indication for a drug, device or nutriceutical already approved for human use

**RAREpositioning**-finding a new rare indication for a human safe pipeline compound

**Commercial**-able to generate a profit for the organization bringing it to market thru FDA approval

**Philanthropic**-able to be used by physicians and patients but unable to generate a profit in the market
RAREpurposing Advantages

• 2000+ generic drugs/3000+ nutriceuticals
• Almost any disease
• Evidence based outcomes
• Fast, cost-effective and widely applicable
• Sometimes clinical before mechanism of action
• Off-label use for philanthropic endpoint
• 505b2 Accelerated Pathway for commercial
RAREpurposing Hurdles

- Generics cheap and widely available
- No/poor IP
  - Repurposing in current dose/delivery
  - MOU patent hard to enforce
- Off-label use
# RAREpurposing Successes

<table>
<thead>
<tr>
<th>Drug</th>
<th>Initial or Intended Indication</th>
<th>Additional Indication</th>
</tr>
</thead>
<tbody>
<tr>
<td>Acetylsalicylic acid (Aspirin)</td>
<td>Inflammation, pain</td>
<td>Antiplatelet</td>
</tr>
<tr>
<td>Amantadine</td>
<td>Antiviral</td>
<td>Parkinson's disease</td>
</tr>
<tr>
<td>Amphotericin B</td>
<td>Fungal infections</td>
<td>Leishmaniasis</td>
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<tr>
<td>Arsenic</td>
<td>Syphilis</td>
<td>Leukemia</td>
</tr>
<tr>
<td>Atomoxetine</td>
<td>Antidepressant candidate</td>
<td>Attention deficit hyperactivity disorder</td>
</tr>
<tr>
<td>Beta-blocker</td>
<td>Arrhythmia/angina</td>
<td>Hypertension, Infantile hemangiomas</td>
</tr>
<tr>
<td>Bevacizumab (Avastin)</td>
<td>Cancer</td>
<td>Macular degeneration</td>
</tr>
<tr>
<td>Bromocriptine</td>
<td>Parkinson's disease</td>
<td>Diabetes mellitus</td>
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<tr>
<td>Dimethyl fumarate (Tecfidera)</td>
<td>Psoriasis</td>
<td>Multiple sclerosis</td>
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<tr>
<td>Doxepin</td>
<td>Antidepressant</td>
<td>Topical antipruritic</td>
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<tr>
<td>Finasteride (Proscar)</td>
<td>Prostate hyperplasia</td>
<td>Hair loss</td>
</tr>
<tr>
<td>Gemcitabine</td>
<td>Antiviral candidate</td>
<td>Cancer</td>
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<tr>
<td>Hydroxychloroquine</td>
<td>Antiparasitic</td>
<td>Arthritis</td>
</tr>
<tr>
<td>Lidocaine</td>
<td>Local anesthetic</td>
<td>Antiarrhythmic</td>
</tr>
<tr>
<td>Lithium</td>
<td>Gout</td>
<td>Bipolar disorder</td>
</tr>
<tr>
<td>Methotrexate</td>
<td>Cancer</td>
<td>Psoriasis, rheumatoid arthritis, medical abortions</td>
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<tr>
<td>Minoxidil (Rogaine)</td>
<td>Hypertension candidate</td>
<td>Hair loss</td>
</tr>
<tr>
<td>Penicillamine</td>
<td>Copper chelating agent</td>
<td>Antirheumatic</td>
</tr>
<tr>
<td>Raloxifene</td>
<td>Breast cancer</td>
<td>Osteoporosis</td>
</tr>
<tr>
<td>Sildenafil (Viagra)</td>
<td>Cardiovascular disorders</td>
<td>Erectile dysfunction, pulmonary hypertension</td>
</tr>
<tr>
<td>Thalidomide</td>
<td>Morning sickness, insomnia</td>
<td>Multiple myeloma, leprosy</td>
</tr>
<tr>
<td>Tretinoin (ATRA)</td>
<td>Severe acne</td>
<td>Leukemia</td>
</tr>
</tbody>
</table>

Adapted from Hahn et al., *Pharmacy Times* 2011
CURRENT FUNDED RESEARCH

30 new projects initiated since August 2015

12 in rare diseases
Including MDS, Batten, myotubular myopathy
pediatric brain cancer (2), CYP24A1

>20 total CWR repurposing projects
in process or recently closed

6 RFPS Launched in 2018 so far, including rare diseases RP, Meniere’s, blood cancers
• 60+ repurposing clinical trials
• 13 “new” repurposed therapies
  • 10 focused on off-label use-8 Rare
  • 3 with commercialization potential
• Average trial cost <$200,000
• Average trial timeline <3 years
RAREpurposing a Transplant Drug Saves Kids with ALPS, a Rare but Deadly Blood Disorder

In 2004, Cures Within Reach funded Dr. David Teachey at Children's Hospital of Philadelphia to repurpose sirolimus, a generic transplant drug, for a pediatric, ultra-rare blood disorder, Autoimmune Lymphoproliferative Syndrome (ALPS). In less than 36 months, Dr. Teachey demonstrated that the drug helped mice with this disease, and then he showed the same with kids who had this disease. 85% of the kids who were treated were in remission after just 90 days on the drug, and these were kids that had failed all other therapies and were slowly dying. Many of these kids have been in remission many years later, taking just two pills each day. Their healthcare costs have gone way down, and they and their families have an almost normal life.

Based on this success in ALPS, in 2011 CWR funded Teachey’s follow-on research to repurpose the same drug and in five additional diseases (Evans disease, systemic lupus erythematosus, autoimmune hemolytic anemia, idiopathic thrombocytopenic purpura and common variable immuno-deficiency) and the same thing happened: 63% kids went into remission and have been living almost normal lives!

KEY FACTS
- CWR funded $73,000 for two projects, in 2004 and 2011, with total project budgets of $78,000

IMPACT
- This treatment is being used for ALPS patients and around the world with great success, as well as in 5 other pediatric autoimmune diseases
- Teachey raised more than $3.25 million in following on funding from the NIH and others
- Teachey published more than 10 articles based on this research
Repurposing Powerful Nutraceuticals for FD, a Rare but Deadly Pediatric Disease

In 2006-8, Cures Within Reach and our fiscal sponsoree FD Now, funded Drs. Berish Rubin and Sylvia Anderson at Fordham University to repurpose various nutraceuticals to help patients with a rare pediatric nervous system disorder, Familial Dysautonomia (FD), caused by a missing protein. Over this period, the researchers discovered that various forms of vitamin A increase production of the protein for these patients, adding to the amount of protein already being increased by earlier discoveries that EGCG, a molecule found in green tea, and tocotrienols, found in Vitamin E, increase the protein. The combination of these three nutraceuticals provided enough protein to significantly reduce the deadly effects of FD, allowing FD patients to live more normal lives. In addition, the researchers discovered an amino acid caused much of the suffering of these children, and created a diet to eliminate this amino acid so that these patients required less protein.

In 2009 FD NOW became its own charity, and over the subsequent years helped these researchers find additional nutraceuticals that now allow these FD patients to replace 100% of the previously missing protein, and these children are now growing into adulthood!

KEY FACTS
- CWR and its fiscal sponsoree FD Now funded $400,000 over two years to support the FD Lab at Fordham University

IMPACT
- FD patients have a life saving regimen of nutraceuticals that restore the missing protein they
- FD Now was supported by CWR and is now an independent charity continuing to support FD research at Fordham.
- FD families have saved significant healthcare costs
<table>
<thead>
<tr>
<th>Disease Area</th>
<th>Institution</th>
<th>Lead Researcher</th>
<th>Name</th>
<th>Type</th>
<th>Other</th>
<th>CWR Project Status</th>
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<tbody>
<tr>
<td>Cardio</td>
<td>Vanderbilt University Medical Center</td>
<td>Dr. Cox</td>
<td>Comparison of Diuretic Combination Therapies in Heart Failure Patients</td>
<td>Drug</td>
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<td>In Process</td>
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<td>Diabetes</td>
<td>Massachusetts General Hospital</td>
<td>Dr. Faustman</td>
<td>Repurposing a Vaccine for Type I Diabetes</td>
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<td>GI</td>
<td>Boston Children's Hospital</td>
<td>Dr. Kahn</td>
<td>Fecal Microbiota Transplantation Patient Registry for pediatric C diff</td>
<td>Other</td>
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<td>Neuro</td>
<td>Johns Hopkins Medicine</td>
<td>Dr. Hollinger</td>
<td>Repurposing a Generic Drug in the Treatment of Pediatric Depression</td>
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<td>Repurposing an Antipsychotic Drug as Treatment for Pediatric Delirium</td>
<td>Drug</td>
<td>Pediatric</td>
<td>Started 2018</td>
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<td>Neuro</td>
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<td>Repurposing Gene Therapy for Batten Disease</td>
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<td>Repurposing a Device to Treat Abnormalities of Gait and Balance in CNS Disorders</td>
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<td>Neuro</td>
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<td>Dr. Oathes</td>
<td>Using Imaging Techniques to Guide Targeted Brain Stimulation in the Treatment of Depression and PTSD</td>
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<td>Dr. Lanctôt</td>
<td>A New Opportunity to Treat Behavioral Problems in Alzheimer’s Patients</td>
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<td>Repurposing a Blood Test to Improve Outcomes for High-Risk Lung Cancer Patients Undergoing Low-Dose CT Screening</td>
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<td>A Phase I Trial for Recurrent Pediatric Brain Cancers Using a Repurposed Generic Drug</td>
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<td>Dr. Suwwan</td>
<td>Low-Level Laser Therapy to Address Treatment Side Effects in Pediatric Cancer Patients</td>
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<td>Combining 9 Repurposed Drugs with a Current Chemotherapy Treatment in Adult Brain Cancer</td>
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<td>Innovations in Combination Therapies for Non-Small Cell Lung Cancer</td>
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<td>Using an Approved Device to Increase the Immune Response in Pancreatic Cancer Patients</td>
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<td>Opthlamic</td>
<td>University of Pennsylvania Medicine</td>
<td>Dr. Kim</td>
<td>Repurposing a Nutriceutical to Treat a Type of Age-Related Macular Degeneration</td>
<td>Nutriceutical</td>
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<td>In Process</td>
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<tr>
<td>Rare</td>
<td>Children’s Hospital of Philadelphia, Hospital for Sick Children</td>
<td>Dr. Levine, Dr. Sochett</td>
<td>Repurposing an Antibiotic to Treat a Defect in Vitamin D Metabolism</td>
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<td>Children’s National Health System</td>
<td>Dr. Nadler</td>
<td>Using a Cancer Drug to Treat a Rare Pediatric Liver Disease</td>
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<td>Pediatric</td>
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<td>Dr. Raza, Dr. Galili</td>
<td>Genetic Sequencing to Identify Treatment Targets in the Rare Blood Disease MDS-RARS</td>
<td>Diagnostic</td>
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<td>Rare</td>
<td>Hospital for Sick Children, National Institutes of Health</td>
<td>Dr. Dowling, Dr. Bonnemann</td>
<td>TAM4MTM: Tamoxifen Therapy for Myotubular Myopathy</td>
<td>Drug</td>
<td>Pediatric</td>
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<td>Vascular</td>
<td>University of Illinois at Chicago</td>
<td>Dr. Havelka, Dr. Eton</td>
<td>Saving Limbs from Chronic Ischemia: Helping Nature Do Its Magic</td>
<td>Combo</td>
<td>Adult</td>
<td>In Process</td>
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</table>
Bioinformatics/Al Impact on Repurposing
PRECISION MEDICINE AND DRUG RAREPURPOSING

• Move from
  • Copying *de novo drug development* methods to using new AI methods
  • Small and large scale trials to N of 1 trials
  • Single drug therapies to combo therapies
  • Static (use till is stops working) to dynamic (keep modifying to keep ahead of the disease) therapy

• Still utilizes the benefits of existing drugs, devices and nutraceuticals
**SEA CHANGE FOR DRUG RAREPURPOSING**

- Computational biology being used to determine how to rarepurpose drugs and nutraceuticals for precision medicine

<table>
<thead>
<tr>
<th>Method</th>
<th>CWR has worked with:</th>
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<tr>
<td>Phenotypic screening</td>
<td>IBM</td>
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<tr>
<td>In silico development</td>
<td>Cyclica</td>
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<td>Gene signatures</td>
<td>Biovista</td>
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<td>Multi-omics</td>
<td>Healx</td>
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<td>GWAS</td>
<td>Notable Labs</td>
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<td>PheWAS/SNPs</td>
<td>OneThree Biotech</td>
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<td>EHR data</td>
<td>Vanderbilt</td>
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<td>Flow Cytometry</td>
<td>Mt. Sinai</td>
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<tr>
<td>Metabolic Modeling</td>
<td>Sanford Burnham</td>
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<tr>
<td>Similarities Modeling</td>
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<tr>
<td>Natural Language Processing</td>
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<tr>
<td>Machine Learning</td>
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DEVELOPING AN AI “TOOLBOX”

<table>
<thead>
<tr>
<th>Method Description</th>
<th>Develop Method</th>
<th>Computational Validation</th>
<th>Experimental Validation</th>
<th>BioPharma Partnership</th>
<th>Accuracy To Date</th>
<th>Publication</th>
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<tr>
<td>Target Deconvolution</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>90%</td>
<td>Madhukar et al, 2017</td>
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<td>Adverse Events</td>
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<td>85%</td>
<td>Madhukar/Gayvert et al, 2018</td>
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<td>Indication Prediction</td>
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<td></td>
<td>82%</td>
<td>Elkhader et al (In Prep) &amp; Madhukar et al, 2017</td>
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<tr>
<td>Analog Optimization</td>
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<td></td>
<td></td>
<td>NA</td>
<td>Prabhu et al, 2017</td>
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<td>Gene Essentiality</td>
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<td>80%</td>
<td>Gilvary et al, 2018 (Submitted)</td>
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<td>Combination Therapy</td>
<td></td>
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<td>84%</td>
<td>Galetti et al, 2018 (Submitted)</td>
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Using genomic data to identify synergistic combinations

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<thead>
<tr>
<th>Gene</th>
<th>SignatureRES</th>
<th>Drug 1 treatment signature</th>
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<tbody>
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</table>
We’ve built a tech-enabled workflow to test more drugs and combinations for each patient sample.

1. Patient consented to trial or tumor banking study
2. Whole Blood from leukemia patient shipped overnight to San Francisco
3. Drugs applied to sample same day using automated lab
4. Therapies ranked by therapeutic index (ratio of targeting leukemia vs. normal cells)
5. Information provided to doctor/pharma partners within 4 days of receiving sample

Why Do Pharma Partners Work With Us?

- **Identify novel combinations**
- **Discover or refine biomarkers** for patient stratification in trials
- **Understand mechanisms** in primary cells with a clinically validated platform
“Transforming the lives of rare disease patients with intelligently matched treatments.”
Applications

- Drug Repurposing ✓
- Combination Therapies ✓
- Target Identification ✓
- Digital Biomarkers ✓
- Therapeutic IP ✓
Digital Biomarkers

Advancing treatments for rare diseases

+8000 metabolic reactions
+5000 metabolites

» Genome
» Transcriptome
» Proteome

Lack of disease understanding

Patient & disease specific 'Omics

Multi-Omic integration

AI optimised genome-scale model

Digital biomarkers & patient stratification

www.healx.io
Drug Development and Drug Repurposing
Driven by Human Biology

Traditional Approach
Drug development lacking human data
- Higher Cost
- Lower Success Rate

In Vitro Experiments
Genetics, Antibody, Small-molecule

Animal Model Experiments
Infer efficacy in humans and conduct trials

Gene/protein drug target of interest

New Paradigm
Drug development driven by human biology
- Higher Success Rate
- Lower Cost

PheWAS takes advantage of naturally occurring variation in humans (SNPs)

Use of proxy SNPs as signals for drug efficacy and safety

Target validation guided by human data supports clinical trial design

Gene/protein drug target of interest

Initial steps to establish a precision indication

PheWAS analysis

Precision indication originates from PheWAS data, is biologically plausible based on evidence, and will move to animal or human validation studies with precise endpoints to confirm mechanism.

Literature review

PheWAS interpretation

Etiology confirmation

Testable hypothesis

Anchoring phenotype and new potential indications

Raw data + evidence

In-depth review of patient medical records

EHRs of associated cases from PheWAS

Databases

PubChem
Genecards
UniProt
OMIM
Drugbank
ETC.

Raw data

Precision indication
Moving straight to human testing when repurposing safe drugs

TITLE: “When Enough is Enough: Decision Criteria for Moving a Known Drug Into Clinical Testing for a New Indication in the Absence of Preclinical Efficacy Data”

Perspective piece in a special issue on drug repurposing in Assay & Drug Development Technologies

Ligand Express - Applications

A structure-based proteome screening platform, leveraging artificial intelligence (AI) for predicting a drug’s effect on proteins, and visualizing the predicted ligand-protein network analysis using systems biology.

- Mechanism-of-Action
- Target Identification
- Adverse Effect Elucidation
- Lead Prioritization
- Drug Repurposing
Validated Results from Ligand Express

Ligand Express generated a novel, testable prediction

PROSPECTIVE VALIDATION:
Drug Repurposing for an Orphan Disease

- **Ligand Express** identified a novel protein in a disease-related pathway for treatment of Systemic Scleroderma

- **Validated in vitro**

- Manuscript **published** in *Arthritis & Rheumatology* (IF 6.9); Cyclica scientists as co-authors
FUTURE OF AI / REPURPOSING?

- **Arivale**: Institute for Systems Biology’s One Hundred Person Wellness Project
- **Goal**: Be your wellness partner for life
- **Pitch**: Combine data, coaching, and individual activity

- **Evaluate**: Genetics, analytes, microbiome, blood and other fluids
- **Capture**: Vitamins, essential fatty acids, kidney and liver function, electrolytes, inflammation, lipids, blood sugar
- **Predict and Respond to**: Obesity, injury risk, aging, organ function, detox, food response/absorption, oral health

- For-profit with 4000 current members hoping to generate big data from 100,000 members
PREDICTION FOR DRUG REPURPOSING

• Each year we will see a doubling or more of the impact of repurposed drugs and nutriceuticals on therapeutics development in rare diseases

• In 5 years we will have turned some acute rare diseases into chronic manageable diseases through precision medicine drug repurposing

• In 10 years, many diseases will be managed as N of 1 ongoing “trials”

• *De novo* drug development will be significantly affected by this trend
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