

PIVOTAL

CAPTISOL-ENABLED, PG FREE, MELPHALAN

MARKET

> 50,000 PATIENTS IN THE U.S. SUFFER FROM MULTIPLE MYELOMA
MELPHALAN (ALKERAN) EXISTING INJECTABLE THERAPY

PRODUCT ADVANTAGES

ALKERAN IS CURRENTLY PACKAGED AS TWO SEPARATE VIALS THAT MUST BE COMBINED PRIOR TO USE AND THEN USED WITHIN 60 MIN OF RECONSTITUTION
PROPYLENE GLYCOL-FREE MELPHALAN FOR INJECTION WILL BE A ONE-VIAL FORMULATION PROJECTED TO HAVE 24 HR USE TIME AFTER MIXING WITH SALINE

DEVELOPMENT STATUS

ORPHAN DRUG DESIGNATION RECEIVED
PATENT APPLICATION FILED MAY 2009
IND FILED, CLINICAL STUDIES INITIATED IN 2010
PROJECTED 505(B)(2) NDA FILING MID 2013

FINANCIAL OPPORTUNITY

ADDRESSES \$80+ MM MARKET
MARKETING EXCLUSIVITY FOR 7 YEARS POST APPROVAL
PDUFA FEE WAIVER (ORPHAN DESIGNATION)

Phase II
Data
Q4 2011

NDA
Filing
Mid-2013

Initiation of
Pivotal
Study
Q1 2012

PHASE II READY

SARM

LGD-4033 Best-in-Class SARM

PRECLINICAL

- Supportive animal toxicity data
- Unique AR binding & selective activity
- Muscle and bone building activity in animal models

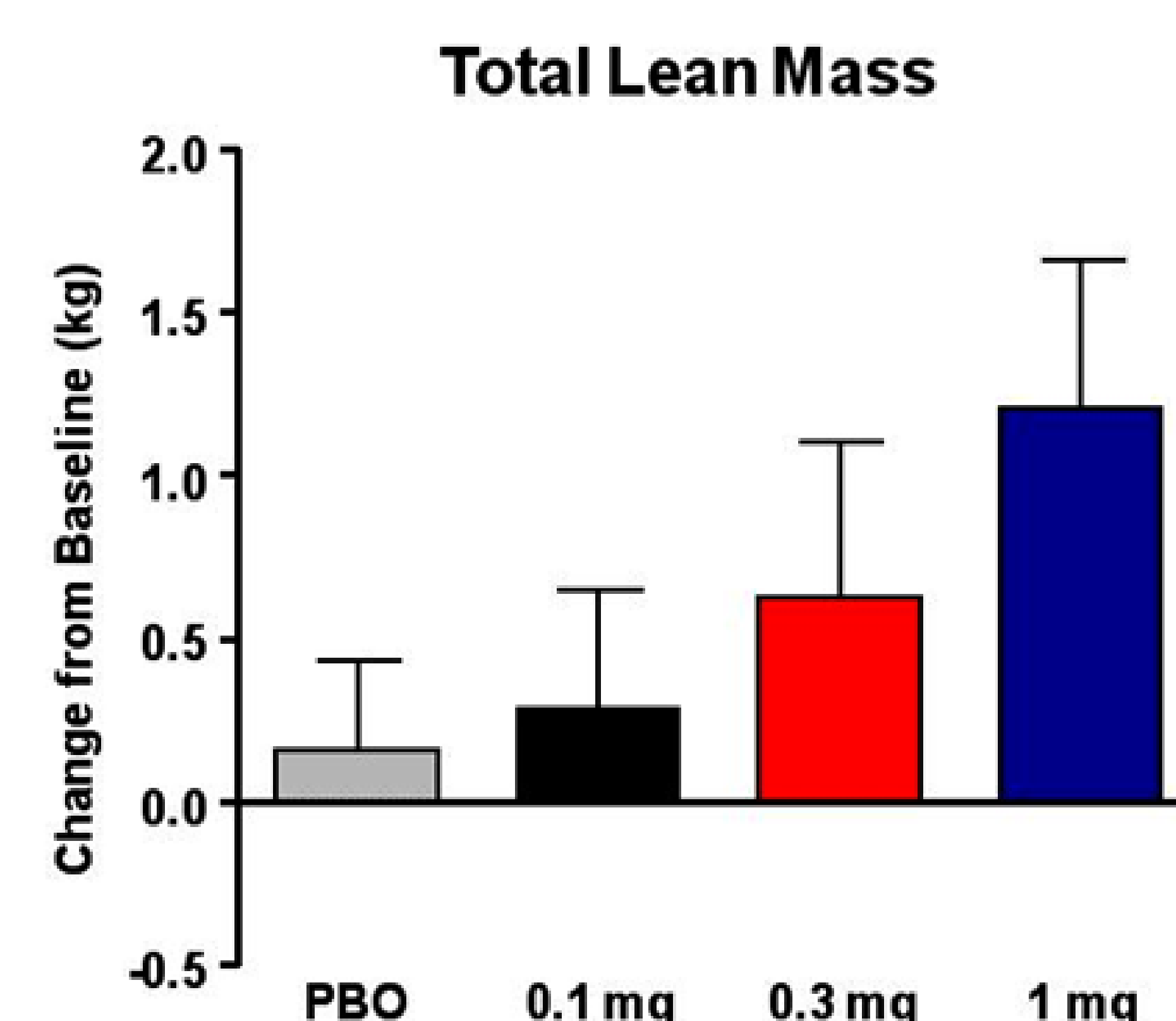
CLINICAL

- Most potent oral SARM
- Ph. I efficacy trends
- Well tolerated with improved safety over anabolic steroids

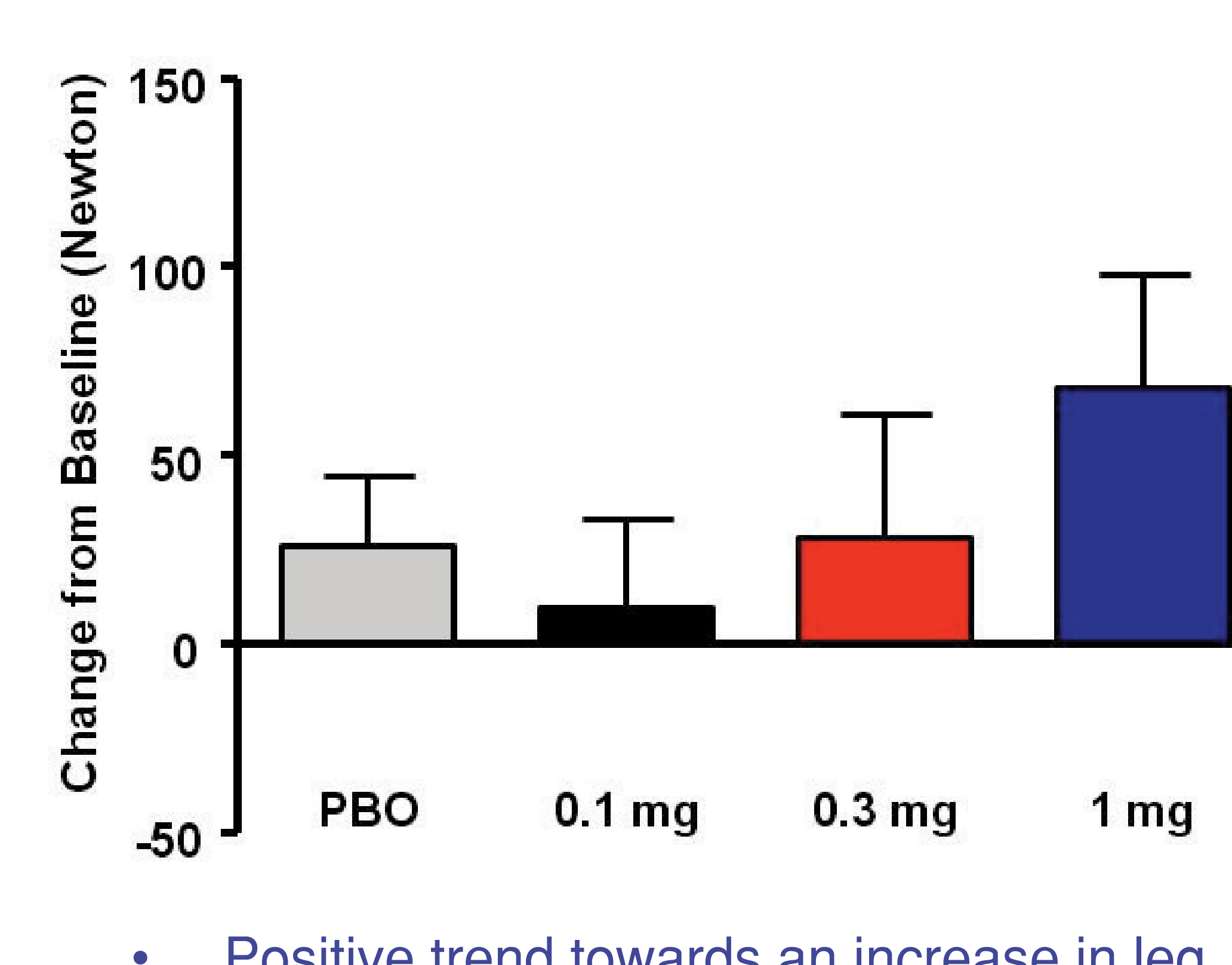
COMMERCIAL

- Potential to address large unmet medical needs in both specialty & long-term muscle wasting patient populations

Lean Mass (kg)
Change from Baseline Up to Day 28



Average Leg Press Force (Newton)
Change from Baseline Up to Day 28



- Positive trend towards an increase in leg press strength with LGD-4033 treatment

PRECLINICAL

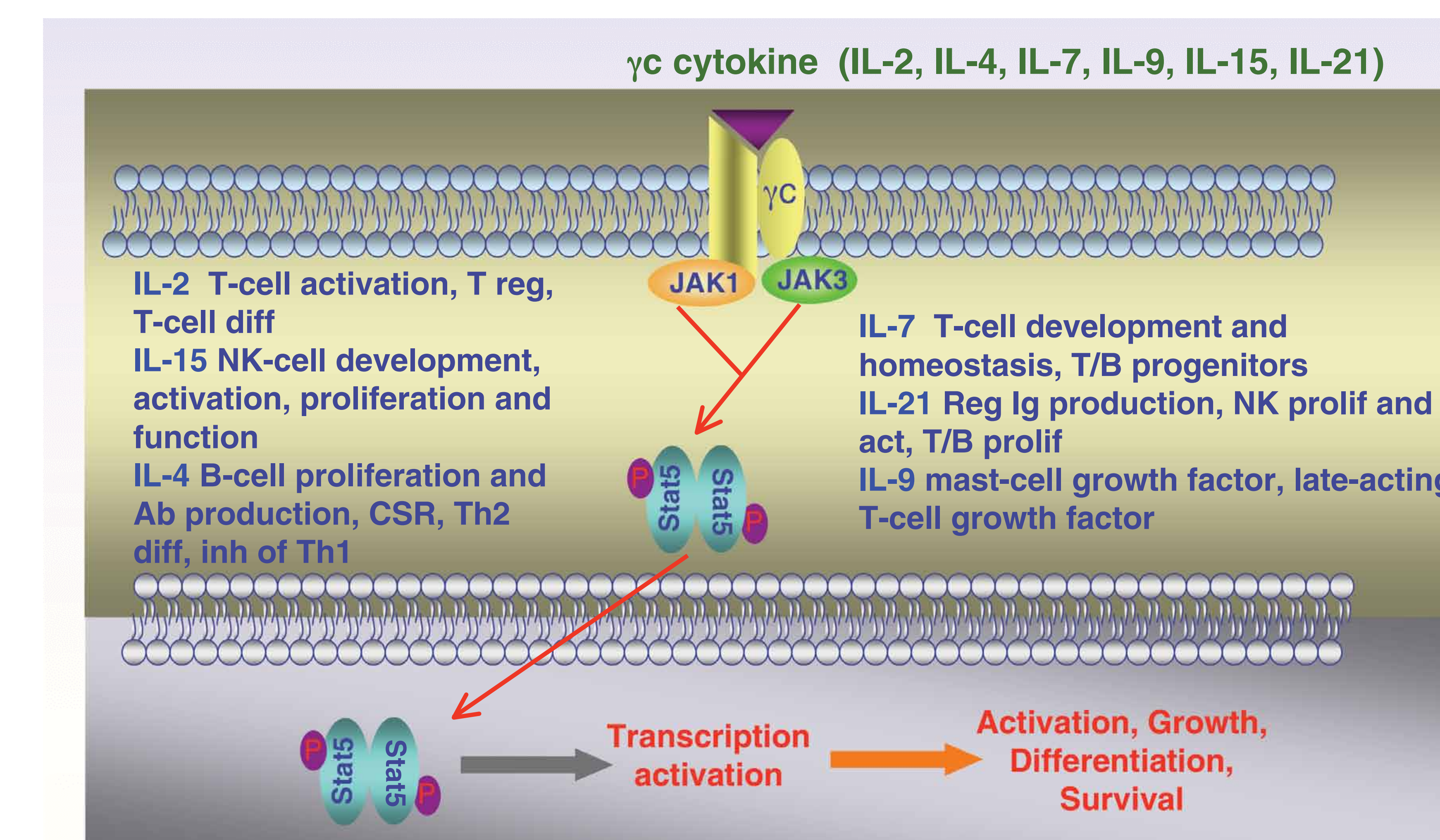
TOPICAL JAK3

TARGET PROFILE

- Small-molecule inhibitors of Janus kinase 3 (JAK3) for the topical or ocular treatment or prevention of skin and eye diseases
 - Specific inhibition of JAK3, which is selectively expressed in immune cells, should provide a lower potential for dose-limiting toxicity than currently available immunosuppressive drugs

JAK3 PROGRAM STATUS

- Ligand retains rights to certain JAK3 compounds developed during an alliance with Wyeth/Pfizer for use in the treatment or prevention of skin and eye diseases
 - Multiple compounds that are potent JAK3 inhibitors (IC50 range: 0.1 – 8 nM)
 - Many with > 10-fold selectivity vs JAK2 and other related kinases
 - Active in cell-based functional assays
 - Compounds identified that are effective systemically or topically in mouse models of JAK3 inhibition
 - No safety issues observed in preliminary studies (i.e. genotoxicity, CYP inhibition, hERG, photocytotoxicity, skin irritation)



DISCOVERY

DIABETES PORTFOLIO

PII FRUCTOSE BISPHOSPHATE (FBP) INHIBITOR

- NOVEL DIABETES MECHANISM OF ACTION
- CLINICAL POC DATA IN HAND

PRECLINICAL GLUCAGON RECEPTOR ANTAGONIST

- NOVEL DIABETES MECHANISM OF ACTION
- HEP-DIRECT DRIVEN LIVER TARGETING MINIMIZES SIDE-EFFECTS

DISCOVERY DGAT INHIBITOR

- NOVEL DIABETES MECHANISM OF ACTION
- POTENTIAL FOR DUAL DIABETES/OBESITY ACTIVITY

DISCOVERY GLUCOKINASE (GK) ACTIVATOR

- NOVEL DIABETES MECHANISM OF ACTION
- LIGAND TISSUE TARGETING TECHNOLOGY

THE BOTTOM LINE

LIGAND'S MELPHALAN IV PROGRAM GIVES LIGAND THE ABILITY TO OWN A PROGRAM THROUGH FDA APPROVAL AND BEYOND WITH MODEST INVESTMENT

THE BOTTOM LINE

LIGAND'S SARM PROGRAM OFFERS OPPORTUNITIES FOR NEW REVENUE THROUGH POTENTIAL LICENSING

THE BOTTOM LINE

THE TOPICAL JAK3 PROGRAM AT LIGAND OFFERS AN OPPORTUNITY TO ENTER THE EMERGING JAK INHIBITION MARKET THROUGH A PARTNERSHIP AND WITH A VERY SELECTIVE MOLECULE

THE BOTTOM LINE

LIGAND'S PORTFOLIO OF DIABETES ASSETS GIVES US THE OPPORTUNITY TO ENGAGE IN A BROAD METABOLIC DISEASE COLLABORATION