

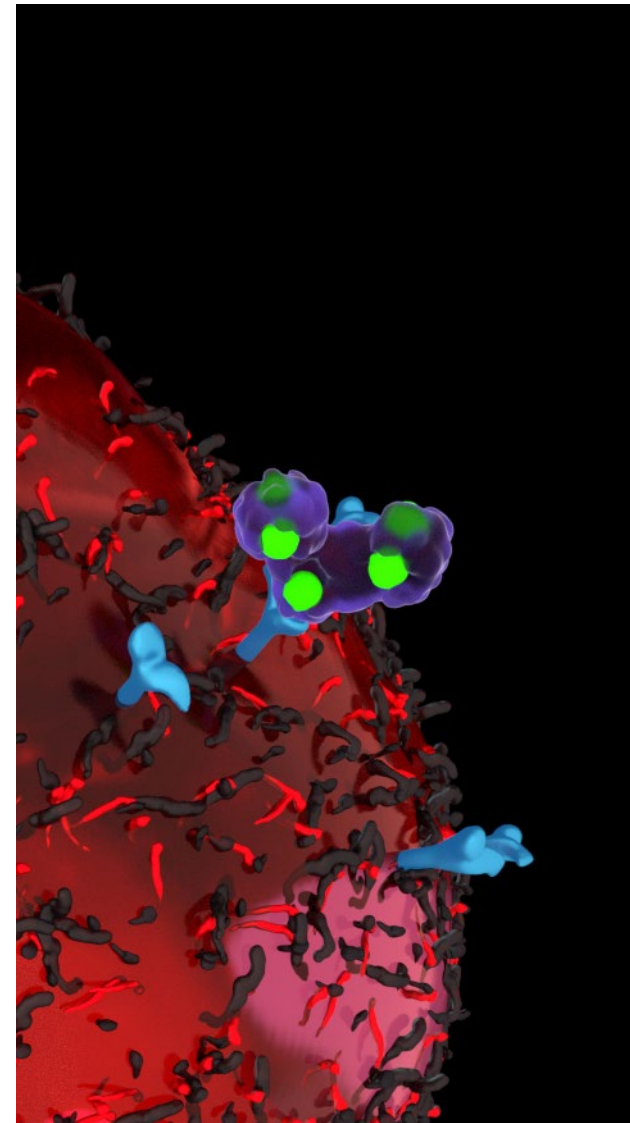
TARGETING CANCER AND AUTOIMMUNE DISORDERS WITH AFP

Dr. Igor Sherman | President & CEO

Richard Potts | Chair

Deltec Annual Conference

March 4-5, 2019



FORWARD LOOKING STATEMENTS

Caution Regarding Forward-Looking Information:

Certain statements contained in this material constitute forward-looking information within the meaning of applicable Canadian provincial securities legislation (collectively, the "forward-looking statements"). These forward-looking statements relate to, among other things, ACT's objectives, goals, targets, strategies, intentions, plans, beliefs, estimates and outlook, and can, in some cases, be identified by the use of words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may" and other similar expressions. In addition, any statements that refer to expectations, projections or other characterizations of future events or circumstances are forward-looking statements. These statements reflect management's current beliefs and are based on information currently available to management.

Certain material factors or assumptions are applied in making forward-looking statements, and actual results may differ materially from those expressed or implied in such statements. Important factors that could cause actual results to differ materially from these expectations include, among other things: uncertainties and risks related to, the availability of capital, changes in capital markets, uncertainties related to clinical trials and product development, rapid technological change, uncertainties related to forecasts, competition, potential product liability, unproven markets for technologies in development, the cost and supply of raw materials, management of growth, effects of payers' willingness to pay for products, risks related to regulatory matters and risks related to intellectual property matters.

When relying on ACT's forward-looking statements to make decisions with respect to ACT, investors and others should carefully consider the foregoing factors and other uncertainties and potential events. Such forward-looking statements are based on a number of estimates and assumptions which may prove to be incorrect, including, but not limited to, assumptions regarding the availability of financing for research and development companies in addition to general business and economic conditions. These risks and uncertainties should be considered carefully and investors and others should not place undue reliance on the forward-looking statements. Although the forward-looking statements contained in this material are based upon what management believes to be reasonable assumptions, ACT cannot provide assurance that actual results will be consistent with these forward-looking statements. ACT undertakes no obligation to update or revise any forward-looking statement.

KEY HIGHLIGHTS

- UNIQUE EFFICACY AND SAFETY PROFILE
- STRONG INTELLECTUAL PROPERTY PORTFOLIO
- ADDRESSES LARGE UNMET MARKET NEEDS
- GREATLY MITIGATED DEVELOPMENT PATHS
- SHORTER PATH TO MARKET– ORPHAN DISEASES
- CLINICAL PLAN – NUMEROUS SHOTS ON NET
- MAJOR SPONSOR – OWNS 15% EQUITY (US\$10.5 MM)
- EXPERIENCED TEAM TO EXECUTE

MANAGEMENT TEAM



Richard Potts Chair

Demonstrated a rich blend of leadership roles with a track record of management, finance and marketing of growth companies across a diverse spectrum of industries, including biotechnology, medical and information technology. Mr. Potts CEO and Chair Lorus Therapeutics (Now Aptose) a TSX/NASDAQ biotech company. He has co-founded, established strategic plans, secured multi-million dollars in financing, public listings and facilitated partnerships as a founder, owner and executive in numerous companies in the knowledge-based industry.



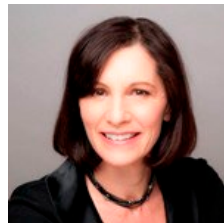
Dr. Igor Sherman President & CEO

Extensive experience and expertise in the pharmaceutical and biotechnology industries, particularly in oncology. Prior to ACT, Dr. Sherman was Director of Clinical Research and Director of Scientific Affairs for YM Biosciences Inc., where he was responsible for preclinical and clinical development, as well as registration strategies for all oncology and pain products in YM Biosciences' portfolio. Dr. Sherman was also Scientific Director of Oncology for AstraZeneca Canada Inc.



Dianne Parsons (CA) CFO

Has held senior financial officer positions focused on strategic planning, operations and financial management in technology-based businesses, turnaround management situations, and manufacturing. She is active in the community and has been an a board member for several non- profit organizations. Ms. Parsons holds a Master of Accounting degree from the University of Waterloo and is a Chartered Professional Accountant and Chartered Accountant.



Betsy Bascom Corporate Development

An entrepreneurial executive with a track record of success in strategic planning, corporate and business development. She is well recognized for her ability to open doors, negotiate and structure strategic alliances and build solid partnerships. Prior to starting her own business, Global Connectworks and working with ACT, Ms. Bascom was the Vice President of Business Development at BIOTEC Canada. She was also the Vice President of Product Development at Umbra Limited and has a Master of Business Administration degree from University of Toronto.



Outsourced Project Management Team Support

COMPANY SUMMARY

ACT IS A CLINICAL STAGE COMPANY WITH PROPRIETARY BIOLOGIC - ALPHA FETOPROTEIN (AFP)

Toronto, Canada-based private company (MaRS Discovery District)



MAJOR MARKET OPPORTUNITIES

- **IMMUNOTHERAPY** | ACT-101 (AFP) for:
 - **Myasthenia Gravis** (muscle weakness) - orphan drug designation in place – Phase II ready;
 - **IBD** (Crohn's/Colitis) – Phase II ready; plus **Hashimoto** Disease, **Multiple Sclerosis**, and 40+ other IgG-driven diseases
- **IMMUNO-ONCOLOGY** | ACT-901 (AFP+Paclitaxel), ACT-902 (AFP+Thapsigargin), ACT-903 (AFP+Linker+Maytansine)
 - Applicable for most solid and liquid tumors
- In-licensed technology with over \$100 million spent on development, demonstrated safety in over 300 patients, international patents in place, large Drug Master file already with FDA including manufacturing, toxicology, and human safety – mitigated development path
- Global Key Opinion Leaders (KOLs) support clinical development plans
- Celgene (Merger with Bristol-Myers Squibb announced January 3, 2019) has invested \$10.5 Million is a 15% equity shareholder with no other commercial rights
- Advancing towards Phase II Myasthenia Gravis trial; Phase II Ulcerative Colitis trial and Phase Ia/b (to efficacy) oncology trial while at same time looking to risk/share, partner or monetize the ACT-101
- US\$18M equity raised to date

THE STORY

HARNESSING NATURE

ALPHA FETOPROTEIN (AFP) IS A HUMAN PROTEIN PRODUCED BY THE EMBRYO DURING FETAL DEVELOPMENT AND SERVES TWO CRITICAL FUNCTIONS:

1. **NORMALIZES IMMUNE SYSTEM** responses so the mother's immune system doesn't attack the embryo. Symptoms of autoimmune diseases: Inflammatory Bowel Disease (Crohn's/Colitis), Myasthenia Gravis, Hashimoto, MS, Arthritis etc. go into remission during pregnancy and this correlates very well with the rise and fall of AFP. AFP safely does this, in part, by interfering with binding of IgGs to FcRn receptors thus reducing levels of toxic IgGs.
2. **PICKS UP NUTRIENTS** from the maternal bloodstream needed by rapidly growing embryo. AFP circulates with the nutrients until it finds receptors which are present on all embryonic cells. It then binds to the receptor and is transported into cell where the payload is released. After birth, the production of AFP stops and the receptors disappear.

Cancer Cells And Suppressor Cells Like Embryonic Cells Express AFP Receptors While Healthy Cells Do Not

By attaching a chemotherapy payload to AFP we can selectively deliver chemotherapy to cancer cells and suppressor cells while bypassing normal cells. This results in increased efficacy by targeting and killing cancer cells and suppressor cells. Killing suppressor cells unleashes the immune system to mount an attack on cancer delivering one-two punch with a significant reduction or no toxicity.

PLANNED CLINICAL USE OF FUNDS

| THERAPY | INDICATION | PARTNERS | DISCOVERY | PRE-CLINICAL | PHASE I | PHASE II |
|---|---|---|-----------|--------------|---------|----------|
| ACT-101 (AFP) | IMMUNOTHERAPY | | | | | |
| | Myasthenia Gravis - ODD (muscle weakness) | | | | | |
| | Inflammatory Bowel Disease (Crohn's/ Colitis) | | | | | |
| | Hashimoto Disease, Multiple Sclerosis, others | | | | | |
| ACT-901 AFP + Paclitaxel | IMMUNO-ONCOLOGY Solid and liquid tumors | Collaboration University Health Network, Princess Margaret Cancer Center | | | | |
| ACT-902 AFP + Thapsigargin | IMMUNO-ONCOLOGY Solid and liquid tumors | Collaboration University Health Network | | | | |
| ACT-903 AFP + Linker + Maytansine | IMMUNO-ONCOLOGY Solid and liquid tumors | Collaboration Polytherics (Abzena) U.K. | | | | |

PRACTICE LEADING PARTNERS



Ensuring efficient cash utilization and a focus on asset development via external (outsourced) support

SCIENTIFIC ADVISORY BOARD



Daniel D. Von Hoff MD, FACP, FASCO, FAACR

Physician in chief and director of translational research at Translational Genomics Research Institute (TGen). He is also a professor of medicine at the Mayo Clinic and medical director of research as well as chief scientific officer at US Oncology. He is most notable for his work in targeted therapies for the treatment of cancer.



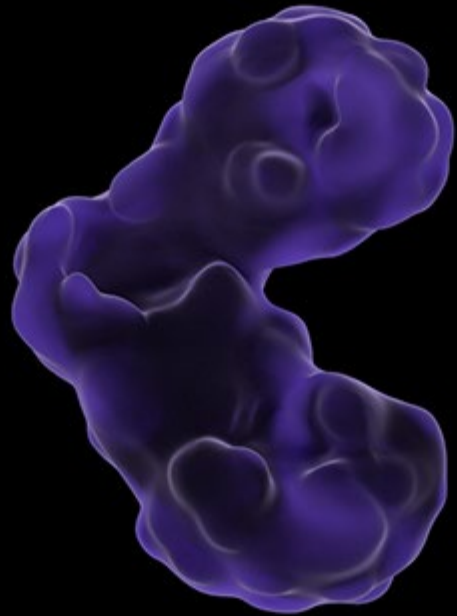
Phil Gold CC, OQ, MD, PhD, FRSC, DSc (Hon) MACP, FRCP(C)

Douglas G. Cameron Professor of Medicine, Professor of Physiology and Oncology, McGill University, Executive Director Clinical Research Centre (MGH) McGill University Health Centre.



Michael Julius PhD

Vice-president, research, Sunnybrook Health Sciences Centre, Vice-president, research, Sunnybrook Research Institute, Senior scientist, Biological Sciences, Odette Cancer Research Program, Sunnybrook Research Institute Professor, department of immunology, University of Toronto



IMMUNOTHERAPY PLATFORM

ACT -101 TO TREAT:

- MYASTHENIA GRAVIS
- IBD CROHN'S/COLITIS
- HASHIMOTO DISEASE
- MULTIPLE SCLEROSIS

ACT-101 OVERVIEW

PRODUCT SUMMARY

ACT-101 IS A SAFE, RECOMBINANT FORM OF AFP IN DEVELOPMENT FOR MODERATE TO SEVERE GENERALIZED MYASTHENIA GRAVIS

OVERVIEW:

- ACT-101 is being developed as an add-on therapy to standard immunosuppressive therapies currently used to treat MG
- ACT-101 is a specific and safe therapy with strong evidence of efficacy in MG

MECHANISM:

- AFP is the third ligand to the FcRn receptor - the key regulator of IgG levels.

PROFILE SUMMARY:

- Very well tolerated molecule with comparable safety to placebo in over 300 patients
 - Safety far superior to existing treatments with respect to both adverse events and ability to add on to primary immunosuppressant regimens
- Expected to demonstrate significant clinical benefit in MG patients

ADMINISTRATION

- Subcutaneous; once weekly

CURRENT LANDSCAPE OF FCRN ANTAGONISTS

Six Molecules in Clinical Development



There are six FcRn antagonists in clinical development today. The most advanced (efgartigimod) is from argenx which has exhibited excellent Phase 2a data in myasthenia gravis and ITP.

While UCB has treated more patients overall it has reported a high rate of headache in its Phase 2a study in Myasthenia Gravis. The other players (Affibody, Alexion, Momenta and Roivant) are all earlier in clinical development.

Obviously, there is a clinical race underway to approvals for relevant IgG-mediated diseases.

COMPARISON OF ACT-101 TO OTHER FCRN ANTAGONISTS

ACT-101 has the potential to be first-to-approval, high value FcRn antagonist. Its MOA is the same as that of the other six clinical candidates. It is subQ which provides for much greater patient convenience. It is ready for commercial manufacture and has an approved Drug Master File. It has an excellent safety record and has been used in over 300 patients (three times as many as the closest competitor). The FDA has suggested that it is ready to enter pivotal studies in myasthenia gravis.

| Sponsor | ACT | argenx | UCB | Momenta | Alexion | Roivant | Affibody |
|-------------------------------|--------------------|--------------|-----------------|-------------|-------------|-------------|-------------|
| Agent | ACT-101 | efgartigimod | Rozanolixizumab | M281 | SYNT001 | HL161 | ABY0389 |
| FcRn Ligand Binding Site | Third (AFP) | First (IgG) | First (IgG) | First (IgG) | First (IgG) | First (IgG) | First (IgG) |
| Clinical Development | MG,RA | MG,ITP,PV | MG,ITP,CIDP | HDFN, MG | PV, WAIHA | MG | NA |
| Most Advanced Completed Study | Phase 2b | Phase 2a | Phase 2a | Phase 1b | Phase 1b | Phase 1a | Preclinical |
| Number of Patients Treated | 300+ | 106 | 128 | 50 | 11 | 0 | 0 |
| Current Mode of Delivery | SubQ | IV | SubQ | IV | IV | IV | SubQ |
| Headache Rate | Similar to Placebo | 25% | 57% | 17% | 55% | NA | NA |

MYASTHENIA GRAVIS CLINICAL ADVISORY BOARD

Phase II Pivotal Study Protocol In Place And Approved By CAB



Vera Bril MD, FRCP

Clinical Researcher, Toronto General Research Institute (TGRi) Clinical Division Head, Toronto General Research Institute (TGRi)

Coordinating investigator in the clinical trial of UCB's rozanolixizumab for MG



James F. Howard Jr. MD

Distinguished Professor of Neuromuscular Disease; Professor of Neurology, Medicine & Allied Health; Chief, Neuromuscular Disorders Section, The University of North Carolina at Chapel Hill. Adjunct Professor of Clinical Sciences (Neurology), North Carolina State University College of Veterinary Medicine.

Lead investigator in the clinical trial of Alexion's Soliris in MG, approved by the FDA in 2017



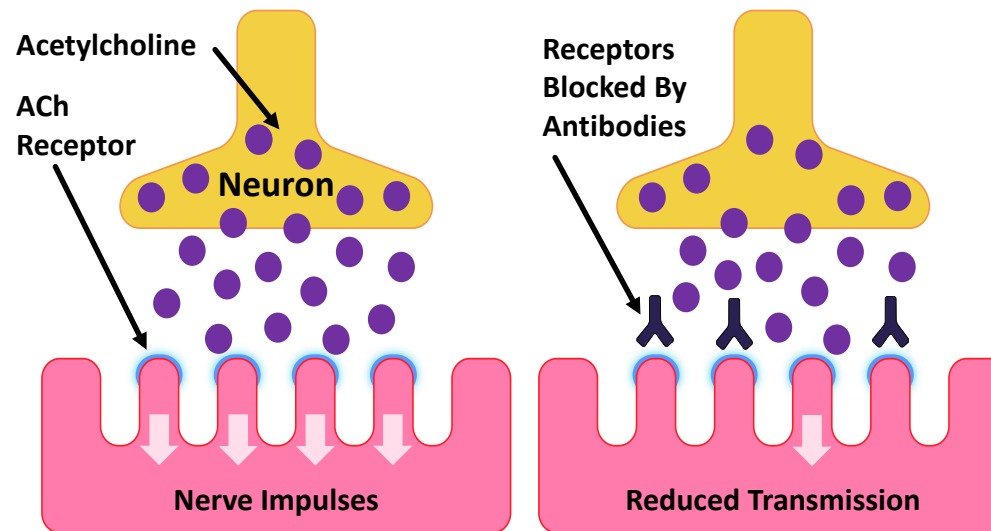
Gil Wolfe MD

Department of Neurology University at Buffalo, State University of New York
Professor and Chairman; Irvin and Rosemary Smith Chair of Neurology

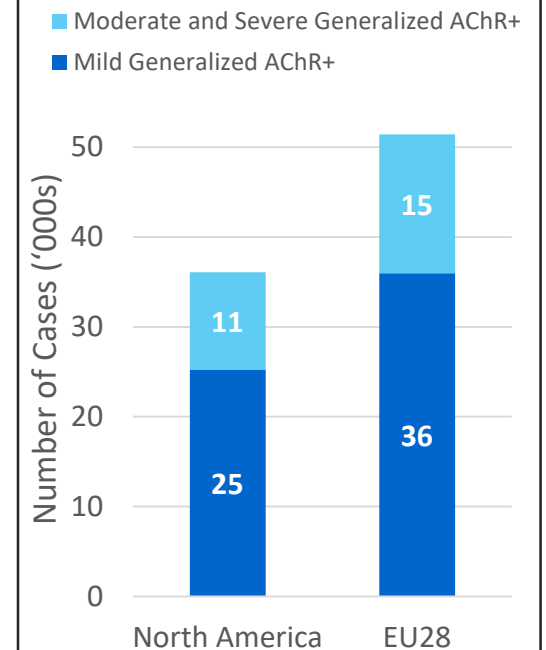
Head of the Panel for Myasthenia Gravis Treatment Guidance

MYASTHENIA GRAVIS (MG) IS AN ORPHAN AUTOIMMUNE DISEASE PRIMARILY CAUSED BY AChR AUTOANTIBODIES

- Myasthenia Gravis is a rare, chronic autoimmune disease affecting an estimated 14/100k individuals. [4,5]
- In over 72% of cases, the impaired neuromuscular function is caused by autoantibodies produced against the AChR receptor which inhibit the action of acetylcholine at neuromuscular junctions.
- AChR autoantibodies gradually destroy the receptors and disrupt the transmission of nerve signals required for control of muscular function.



**Generalized AChR+ MG
Prevalence by Severity [6]**



ACT-101 OVERVIEW PRECLINICAL EFFICACY (1)

TREATMENT WITH ACT-101 HAS REDUCED DISEASE SEVERITY AND LOWERED ANTI-AChR ANTIBODIES LEVELS IN PRECLINICAL MG MODELS

EXPERIMENTAL DESIGN

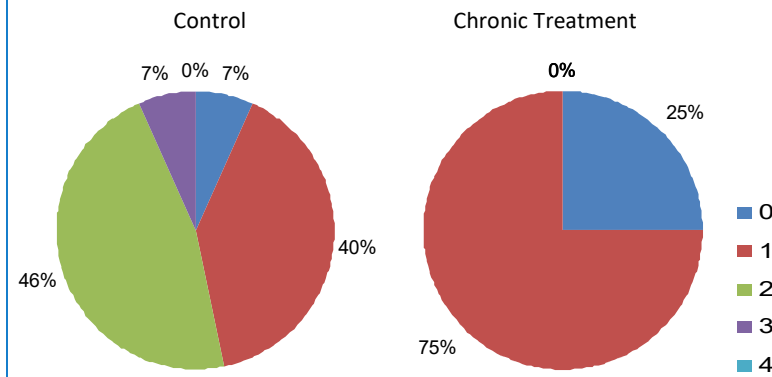
- Female Lewis rats with experimental autoimmune myasthenia gravis (EAMG) induced by injections of Ach-torpedo preparation were treated with alpha-fetoprotein to explore the therapeutic value of AFP
 - Control Group: saline (n=26)
 - Chronic Phase Group: rhAFP 60µg/kg/day from day 26 post-induction (n=26)
- Sera from the rats were assayed for anti-rat AChR antibodies and the rats were given a clinical severity score 0-5 based upon inspection for muscle weakness
- Dosage chosen to mimic AFP level during remission of autoimmunity in the 2nd half of human pregnancy

RESULTS

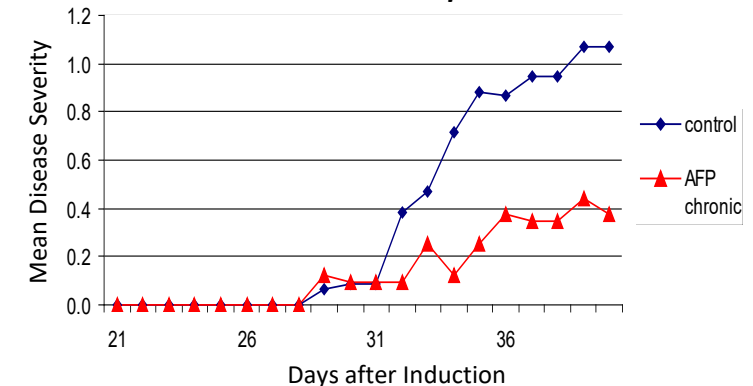
- Statistically significant reductions in disease severity and incidence were observed following rhAFP treatment
 - EAMG rats treated with AFP showed normal response to repetitive nerve stimulations, compared to placebo-controlled EAMG rats who showed a decreased response
 - Levels of anti-AChR antibodies directed against rat-AChR were lowered by 62% in the treated rats
- In the Chronic Phase Group, the natural course of gradual disease progression did not occur

SELECT STUDY DATA

Distribution of Maximal Disease Severity Score



Chronic Treatment Group Versus Control



ACT-101 OVERVIEW

PRECLINICAL EFFICACY (2)

IN A SUBSEQUENT STUDY, ACT-101 LOWERED CLINICAL SCORE, INCREASED GRIP STRENGTH, AND REDUCED IgG2 LEVELS IN MG MODELS

STUDY DESIGN

Objective: To evaluate the efficacy of ACT-101 in an experimental autoimmune MG rat model

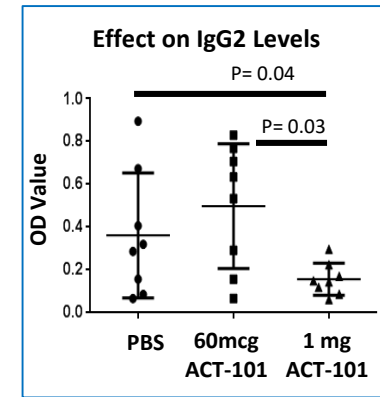
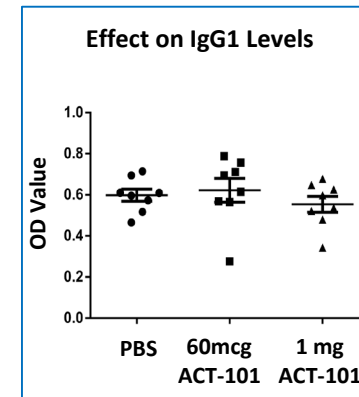
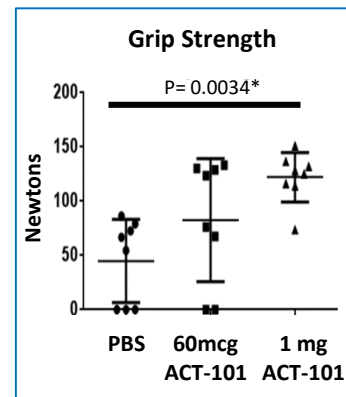
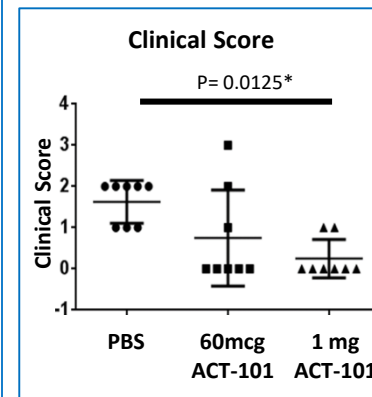
Experimental Design

- Experimental autoimmune MG was induced in 25 Lewis Female rats with tAChR
- On day 40 after induction, daily intraperitoneal injections of ACT-101 (60 mcg or 1 mg) or equivalent volume of placebo (PBS) was initiated
- After 14 days of treatment, EAMG rats were euthanized and samples were taken

RESULTS SUMMARY

- EAMG rats that received 1 mg of ACT-101 demonstrated lower clinical scores and greater grip strength than control rats
- Complement-activating anti-AChR IgG2 levels were significantly lower in AFP-treated rats while non-complement activating IgG1 levels were not reduced

SELECT STUDY DATA



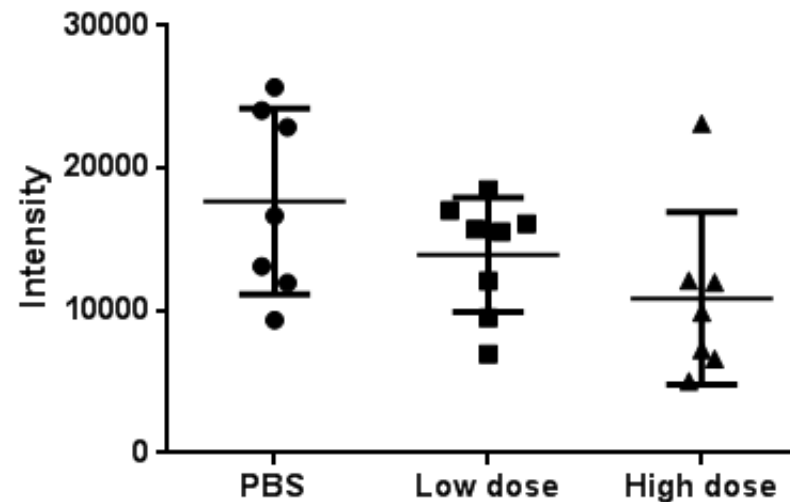
*2-way ANOVA

SUBSTANTIAL
IGG
KNOCKDOWN
SEEN

The amount of IgG antibodies bound to Ach receptors in the gap junction was significantly lower in AFP-treated rats compared to controls

SELECT STUDY DATA

IgG AFP EAMG



ACT-101 OVERVIEW CLINICAL SAFETY DATA

**THE DOSE OF ACT-101 PROVEN SAFE IN CLINICAL STUDIES IS ~3 TIMES
HIGHER THAN THE SERUM LEVEL REQUIRED TO ACHIEVE AN EFFECT**

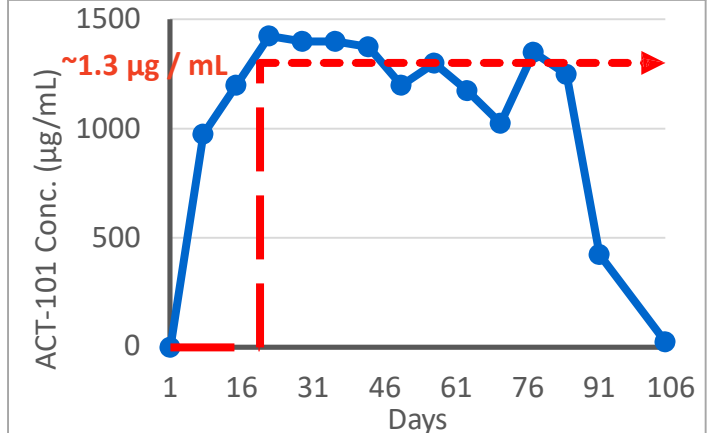
SAFETY STUDIES

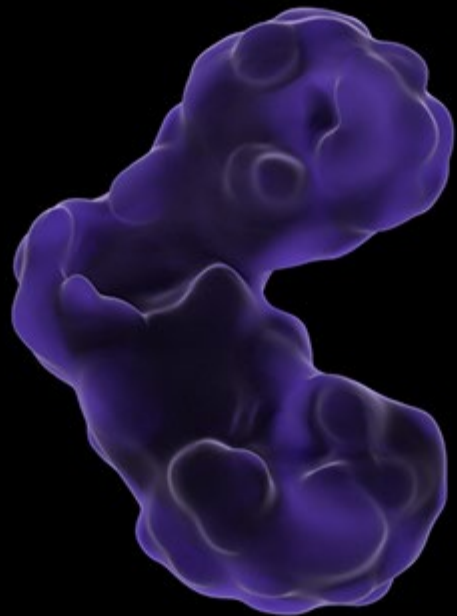
1. Experimental binding studies show AChR autoantibody binding inhibition by AFP of approximately 74% at an AFP concentration of 0.4 $\mu\text{g/mL}$
 - This data follows the observation that serum concentration level in pregnancy (0.3 – 0.5 $\mu\text{g/mL}$) are associated with remission
 2. ACT-101 has demonstrated a strong safety profile across six clinical studies up at doses from 12 to 100 mg
 - ACT-101 was previously being developed through Phase II by Merrimack Pharma for RA, psoriasis, and uveitis
 - Over 300 patients, treated for up to 24 weeks, demonstrated good tolerability
- ❖ Antibodies to ACT-101 were not detected in any of these studies

DOSING

Based on the robust safety data, dosing of ACT-101 can safely exceed the serum concentration suggested to achieve clinical remission

ACT-101 SERUM LEVELS AFTER 21 MG SUBCUTANEOUS INJECTION





IMMUNOTHERAPY PLATFORM

ACT - 101 EXTENSION TO:

- IBD (CROHN'S/COLITIS)
- HASHIMOTO DISEASE
- MULTIPLE SCLEROSIS
- 40+ OTHER AUTOANTIBODY DISEASES

IBD

CROHN'S & COLITIS

CLINICAL ADVISORY BOARD



Jean-Frédéric Colombel -MD

Head of the Center of Inflammatory Diseases of the Intestine at the Department of Gastroenterology of Icahn School of Medicine at Mount Sinai in New York. Professor of Gastroenterology & Hepatogastroenterology and Director of the Department of Hepatogastroenterology in Hopital Huriez, CHRU Lille (FRA). Chairman and President of the European Crohn's and Colitis Organization, and Scientific Secretary of the International Organization of IBD (IOIBD).



Pierre Desreumaux, MD, PhD

Professor of Gastroenterology, PhD in Immunology at the University and Medical School of Lille, France. Clinician in the Department of gastroenterology and nutrition of the university hospital of Lille, he is also head of the Research Unit on the patho-physiology of Inflammatory Bowel Diseases (IBD)



Stephen B Hanauer, MD

The Clifford Joseph Barborka Professor of Medicine in the Division of Gastroenterology and Hepatology at Northwestern University, Chicago. His clinical and research focus is in Crohn's disease and ulcerative colitis.

ADDITIONAL INDICATIONS

IBD RATIONALE AND PRECLINICAL EXPERIMENTAL DESIGN

ACT CONDUCTED A PRECLINICAL ANIMAL STUDY TO FURTHER SUPPORT THE CLINICAL RATIONALE FOR THE USE OF ACT-101 WITHIN IBD

THERAPEUTIC RATIONALE

- AFP's utility in IBD stems from experimental findings that the compound reduces the ability of dendritic cells to present antigens and downregulates the production of antibodies by B cells
- A randomized clinical study conducted in Russia and Israel between 2000 and 2004 explored the potential of AFP in treating ulcerative colitis and Crohn's disease in 78 patients (56 with colitis, and 22 with Crohn's, 50:50 randomization)*
 - After treatment with AFP, 100% of patients experienced definite improvement in their symptoms
 - 30% of Crohn's patients and 32% of colitis patients had complete restoration of normal bowel movements and no symptoms of disease post treatment
 - 30% of Crohn's patients reduced steroid dose by half and 20% went off steroids completely; 32% of colitis patients were able to reduce steroid dose by 40-60% and 7% went off steroids completely
 - No significant changes were observed in the placebo group
- Alpha Cancer conducted pre-clinical studies to validate the potential of AFP in a model of IBD



PRECLINICAL STUDY DESIGN

- Colitis was induced by anesthetizing mice and administering intrarectal 2,4,6-Trinitrobenzenesulfonic acid (TNBS)
- Macroscopic and histological assessments of colitis according to Wallace criteria performed blindly by two investigators
- Ameho's score, MPO, TNF α , IFN γ , IL-1 β , and IL-10 in colon tissues were also measured
- Mice were divided into the following groups:
 - 1) Vehicle (n=19)
 - 2) AFP 100 μ g/day SC (n=19)
 - 3) AFP 200 μ g/day SC (n=19)
 - 4) AFP 300 μ g/day SC (n=19)
 - 5) AFP 200 μ g/day IV (n=19)
 - 5) Positive Control - Anti-TNF α (n=19)

ADDITIONAL INDICATIONS

IBD PRECLINICAL
EXPERIMENTAL RESULTS:
WALLACE'S SCORE

PRECLINICAL RESULTS OF ACT-101 IN A "GOLD STANDARD" MODEL OF IBD SHOWED A 53% DECREASE IN Ameho's SCORE WITH RESULTS SUPERIOR TO ANTI-TNFα

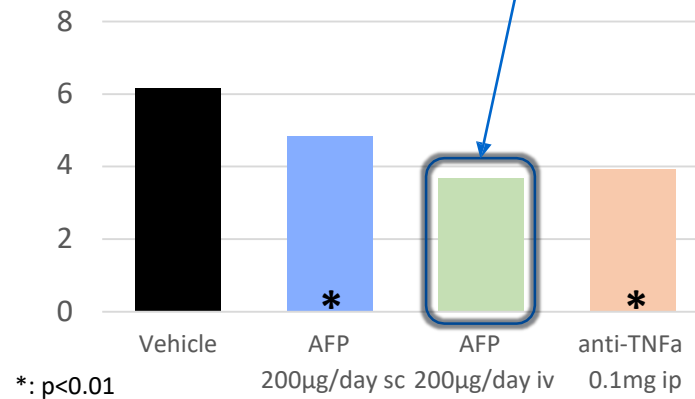
RESULTS: MACROSCOPIC AND HISTOLOGIC EVALUATION

- A significant decrease in inflammation at the macroscopic level was observed with AFP at 200μg and 400μg/mouse/day administered by sc or iv injection
- A strong correlation between macroscopic and histological scores (Wallace's Score and Ameho's Score) show a significant improvement in inflammation

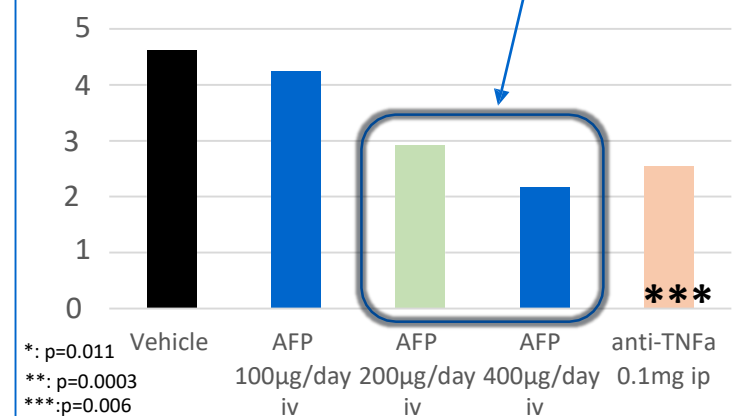
41% decrease with AFP 200μg/day by IV administration (similar effect to anti-TNFα)

53% decrease in inflammation at the histological level than anti-TNFα at 45%

Wallace's Score



Ameho's Score

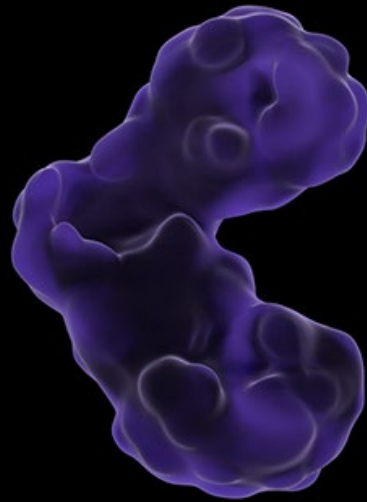


COMMERCIAL POTENTIAL

PRECEDENT ACQUISITIONS:

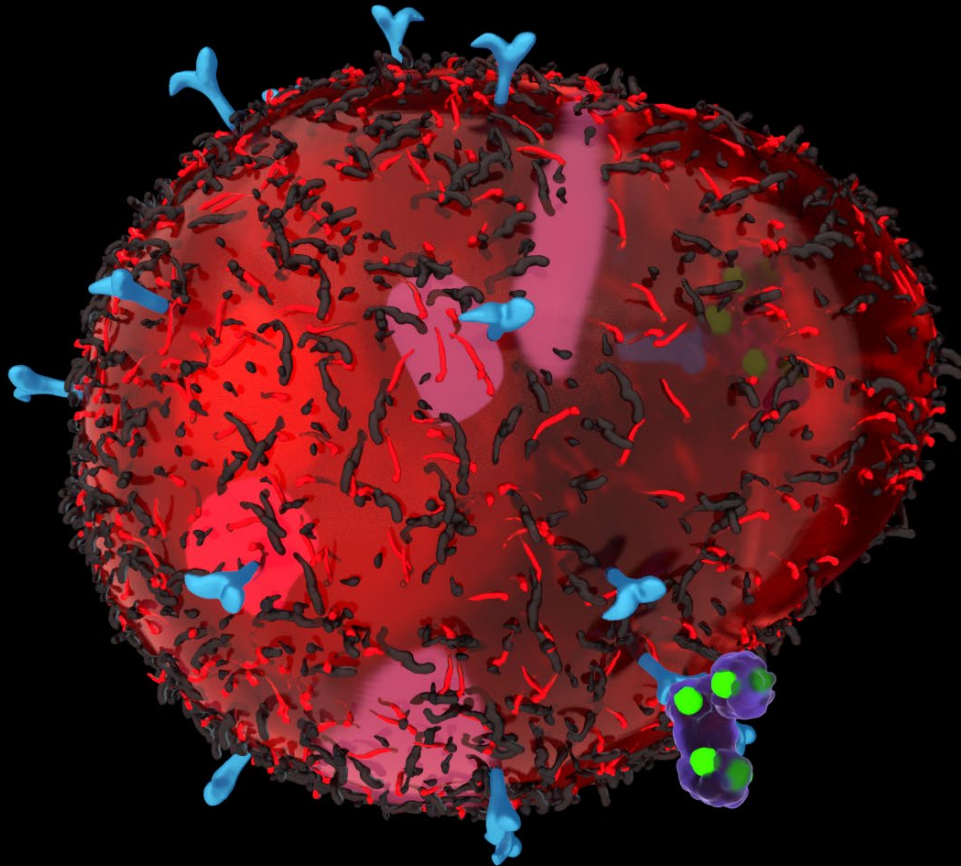
- ✓ Roche buys Adheron - Phase I (2015) **\$580M**
- ✓ Bristol Myers buys Padlock - Preclinical (2016) **\$600M**
- ✓ Celgene buys Delinia – Preclinical (2017) **\$775M**
- ✓ J&J buys Theravance - Phase I (2018) **\$1Billion**
- ✓ Alexion buys Syntimmune – Phase I (Sept 2018) **\$1.2Billion**

BUSINESS DEVELOPMENT SUMMARY



IMMUNOTHERAPY PLATFORM

- ✓ Novel anti-inflammatory immune modulating agent
- ✓ Protein fully characterized
- ✓ Mechanism of action clear
- ✓ Massive safety data in over 300 humans – non-immunogenic
- ✓ International patents in place to 2025+ and additional 7/12 years market and data exclusivity as a biologic
- ✓ Phase II Ready in two indications
- ✓ Clinical Advisory Boards set – Orphan Designation in place
- ✓ Confirmation of efficacy in animal models
- ✓ FDA approved manufacturing process in place
- ✓ Significant risk mitigation and short path to value inflection
- ✓ **Monetization/Partnering efforts under way**

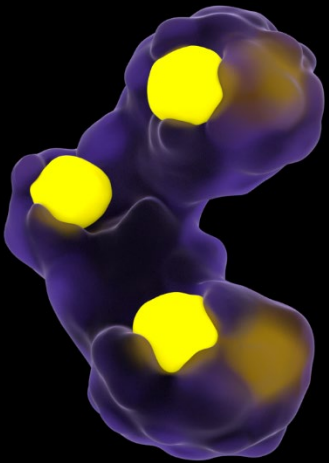


TARGETED IMMUNO-ONCOLOGY DELIVERY PLATFORM

ACT-901, 902, 903

LEAD ASSETS

ACT-901, 902, 903



ACT-901 (AFP + PACLITAXEL), **ACT-902** (AFP + THAPSIGARGIN), **ACT-903** (AFP + LINKER + MAYTANSINE)

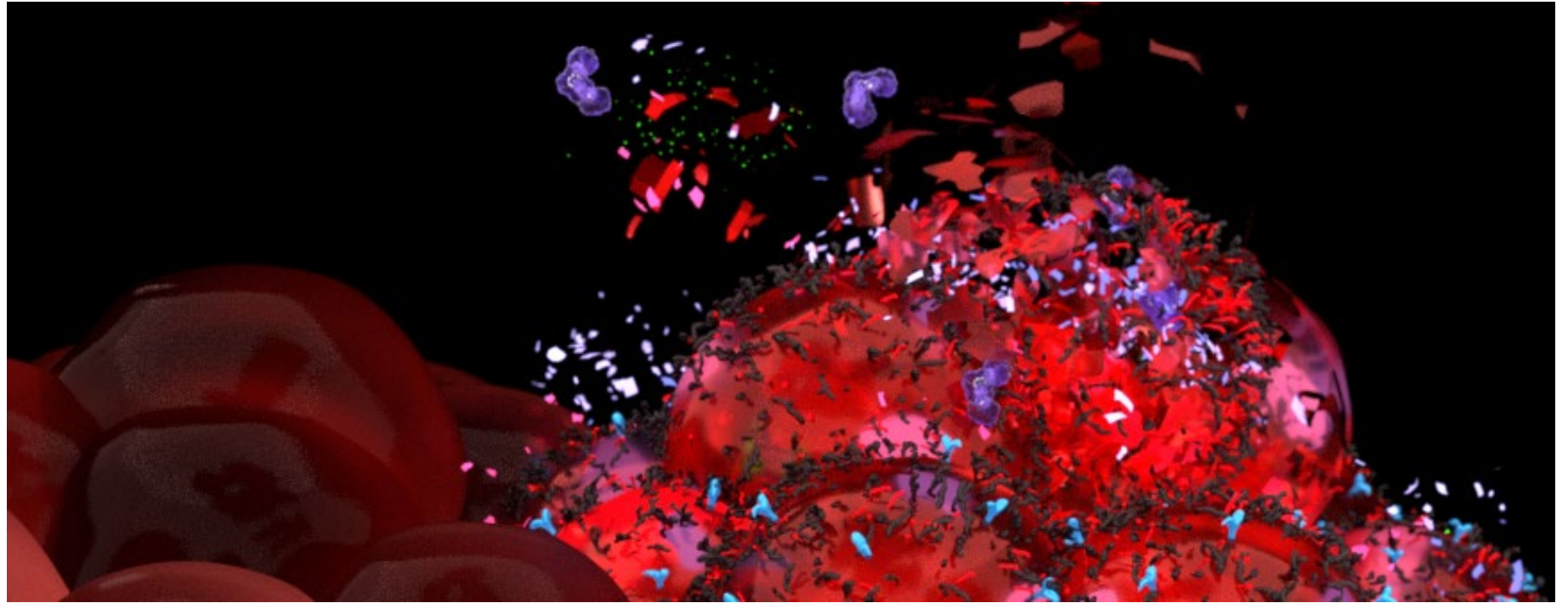
- AFP is a shuttle protein that targets AFP receptors on cancer cells
- Majority of solid and liquid cancer cells (>80%) have AFP receptors
- **Healthy adult cells do not have AFP receptors**
- Combines highly targeted transporter protein AFP with a chemotherapy payload – could be generic or proprietary
- This chemotherapy payload is delivered selectively to cancer cells

BENEFITS OF ACT'S TARGETED APPROACH

- Formulation combines two well-known molecules
- Currently in development for testicular germ cell tumors – will seek Orphan Drug / Breakthrough Therapy Designation
- Will expand to other major indications
- Overcomes chemotherapy drug resistance as delivery bypasses membrane pumps
- AFP is non-immunogenic in humans – demonstrated safety in over 300 patients (as safe as placebo)
- Frequency of treatment driven by efficacy, not toxicity avoidance
- Treatment expected to minimize pain, suffering and healthcare cost

TARGET PRODUCT PROFILE

AFP + CHEMOTHERAPY



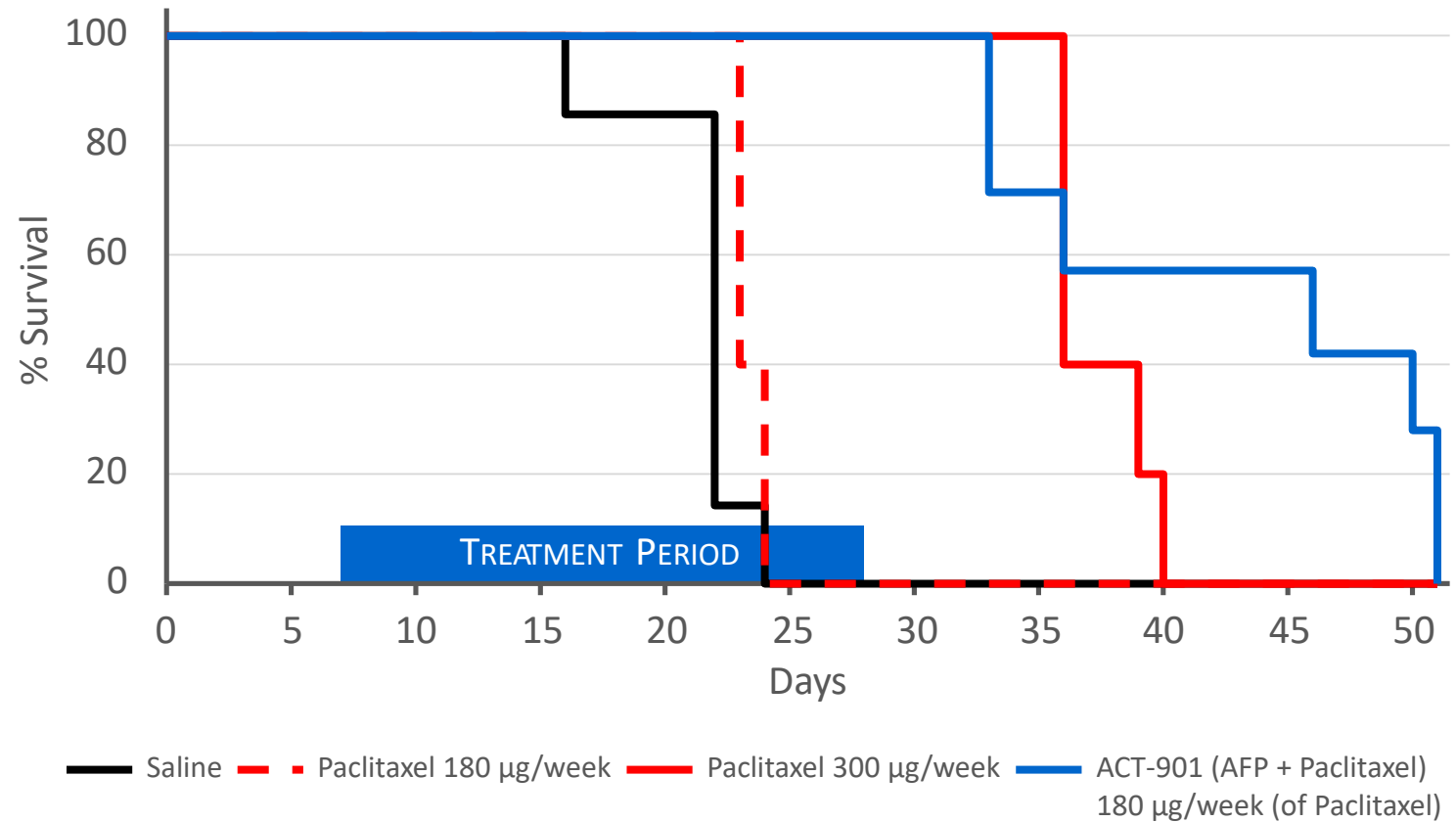
- Significantly less toxicity than targeted and non-targeted chemotherapy
- Improved efficacy compared with chemotherapy, targets cancer and stimulates immune system
- Not necessary to preselect patients for expression of the AFP receptor, because receptors are expressed in most cancers (solid and liquid), but are absent in normal tissues
- No neutralizing antibodies are triggered by AFP – fully human protein

EVIDENCE OF EFFICACY

IN VIVO SURVIVAL RESULTS
ACT-901 (AFP +
PACLITAXEL)

UHN – PRINCESS MARGARET CANCER CENTRE

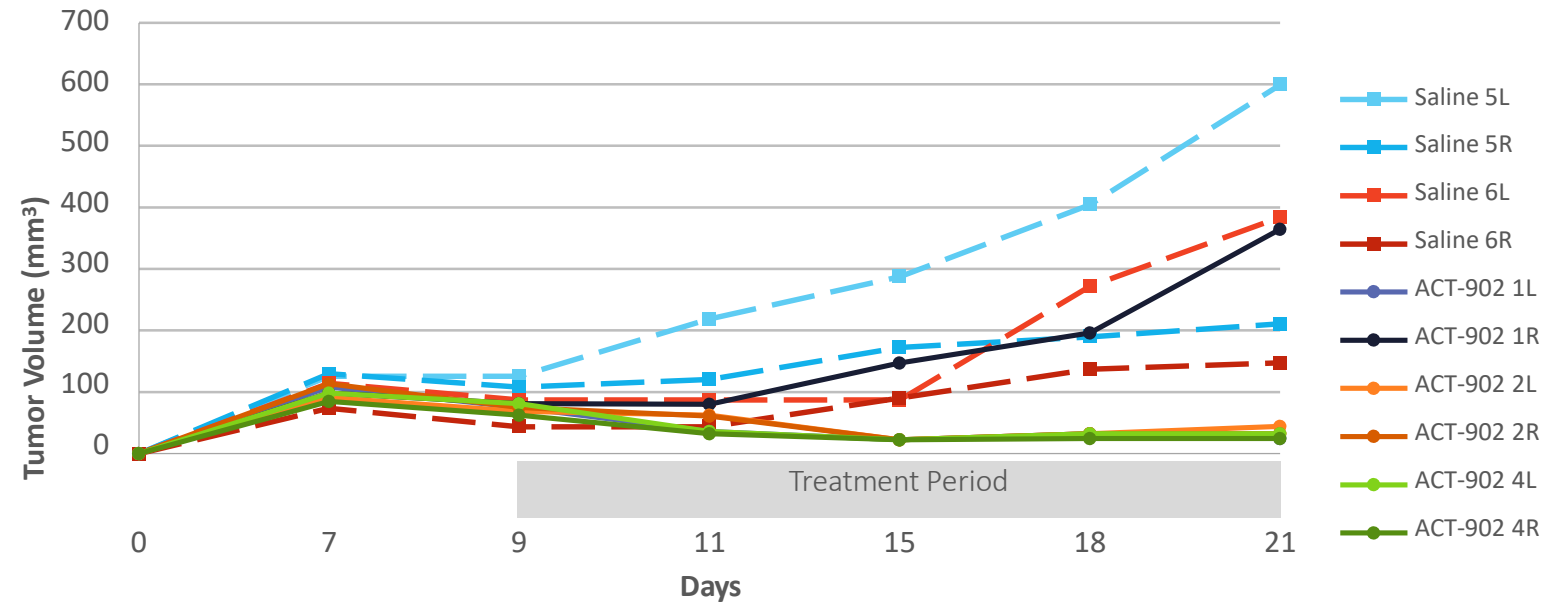
In Vivo Survival Results



EVIDENCE OF EFFICACY

IN VIVO STUDY
ACT-902
(AFP + THAPSIGARGIN)

PRIMARY HUMAN CANCER CELL LINE POP-92 XENOGRAFT MODEL



In this study 2 groups of mice were treated with saline (control group, n=4) or ACT-902 (n=6). Dotted lines show individual tumor volumes in control mice. All mice were dead by day 24. Solid lines show changes in tumor volumes of mice treated with ACT-902.

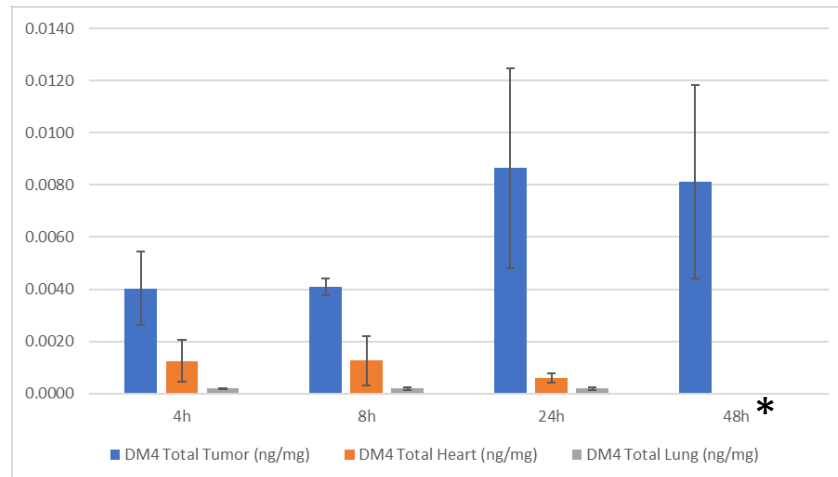
5 out of 6 ACT-902 treated tumors show complete regression of tumors by day 7 of treatment with no further growth thereafter. One tumor was unresponsive and continued to grow.

It should be noted that POP-92 cancer cells are from patient with BRAF mutated colorectal cancer. This type of cancer is highly resistant to chemotherapy resulting in poor prognosis for patients with this cancer type.

ACT-903 - Delivery is Selective for Tumor

Bone Marrow Toxicity - Below Detection Level

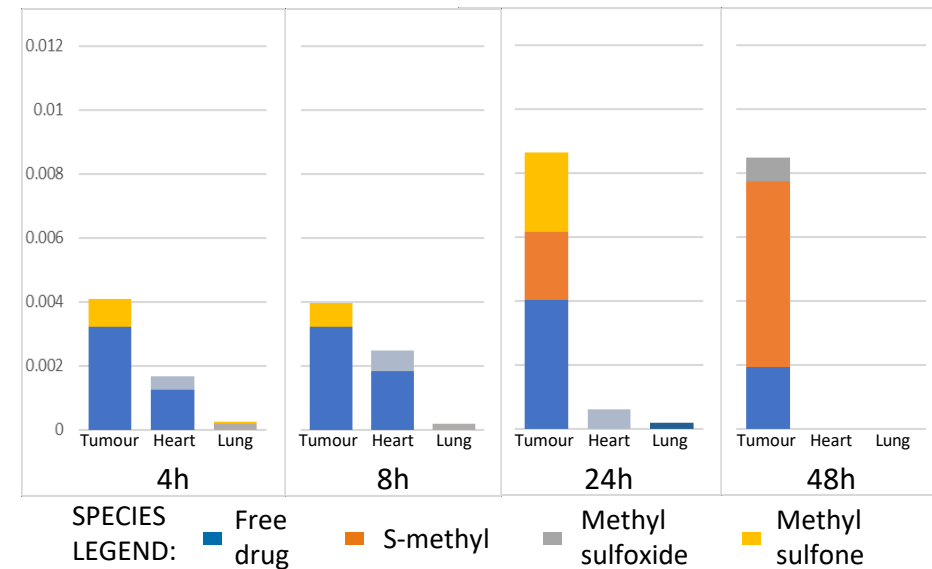
Total free drug + metabolite concentration at
4h/8h/24h (ng/mg)



| | DM4 Total Tumour (ng/mg) | DM4 Total Heart (ng/mg) | DM4 Total Lung (ng/mg) |
|------|-----------------------------|----------------------------|---------------------------|
| 4h | 0.0040 | 0.0013 | 0.0002 |
| 8h | 0.0041 | 0.0013 | 0.0002 |
| 24h | 0.0086 | 0.0006 | 0.0002 |
| 48h* | 0.0081 | n/a | n/a |

* Tumour only data available, n =2

Free drug and metabolite concentration at
4h/8h/24h (ng/mg)



AFP TARGETS FOUND ON TUMOR SUPPRESSOR CELLS

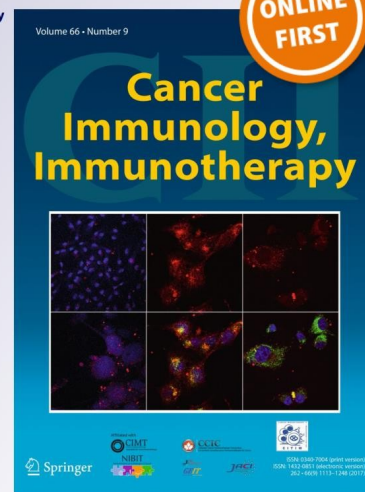
Daunorubicin conjugated with alpha-fetoprotein selectively eliminates myeloid-derived suppressor cells (MDSCs) and inhibits experimental tumor growth

Nikolai N. Belyaev, Nurshat Abdolla, Yuliya V. Perfilyeva, Yekaterina O. Ostapchuk, Vladimir K. Krasnoshtanov, Aikyn Kali, et

Cancer Immunology, Immunotherapy

ISSN 0340-7004

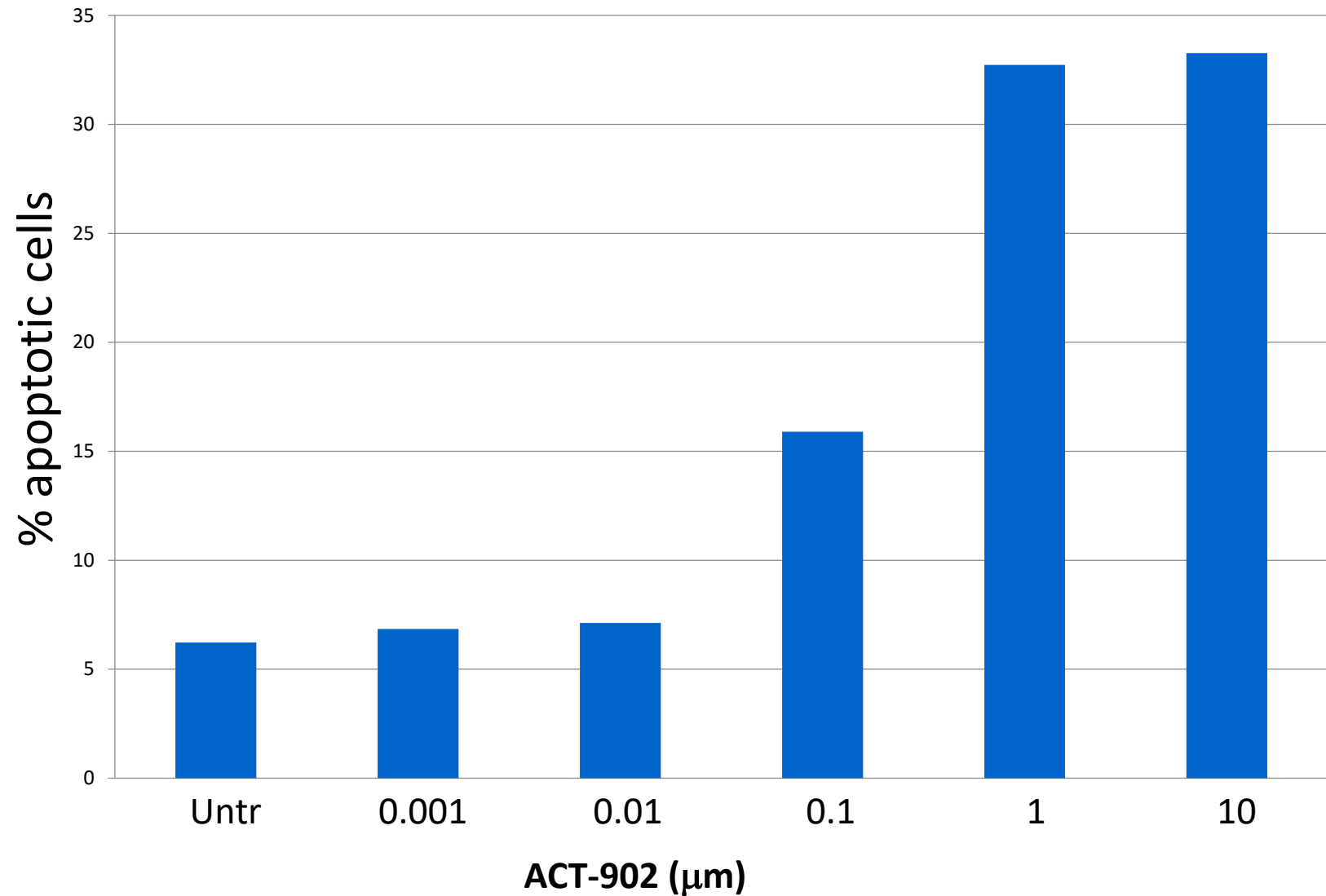
Cancer Immunol Immunother
DOI 10.1007/s00262-017-2067-y



 Springer

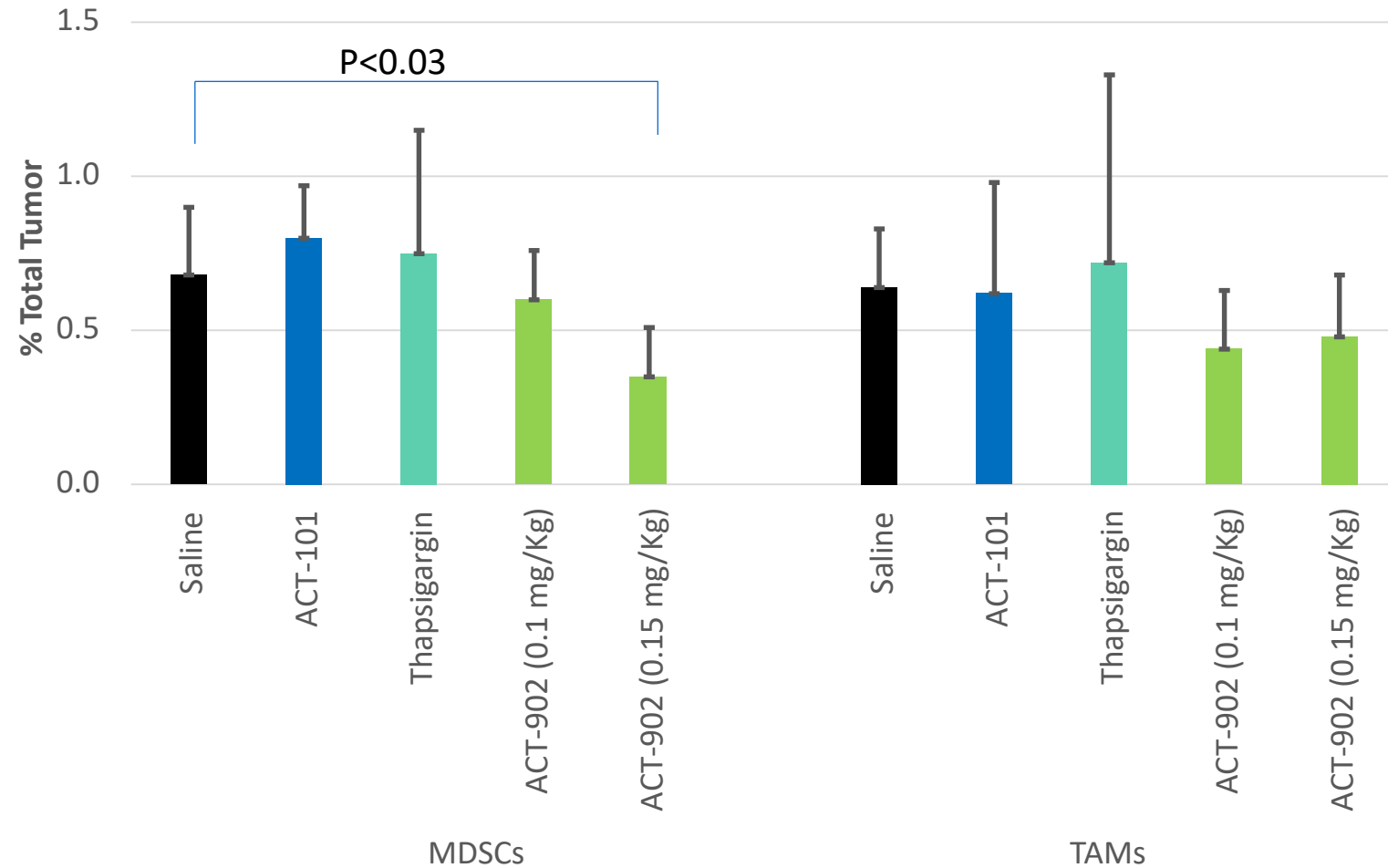
DOSE-DEPENDENT KILLING OF MDSCs

MDSC Cells Incubated in Various Concentrations of ACT-902



EFFECT OF ACT-902 ON TUMOR- INFILTRATING MDSCs

**% of MDSCs And TAMs In Tumors From Various Treatment Groups
After 4 Days Of Treatment**



MITIGATED

DEVELOPMENT, MANUFACTURING, AND REIMBURSEMENT RISKS

EFFICACY

- ✓ Use of well studied effective chemotherapies widely used in multiple forms of cancer
- ✓ Enhanced efficacy in multiple tumor cell lines and xenograft models already demonstrated

SAFETY

- ✓ ACT-101 (targeting protein) shown to be as safe as placebo in over 300 humans in Phase I/II clinical testing
- ✓ Chemotherapy safety profiles well understood

DEVELOPMENT PATH IS CLEAR

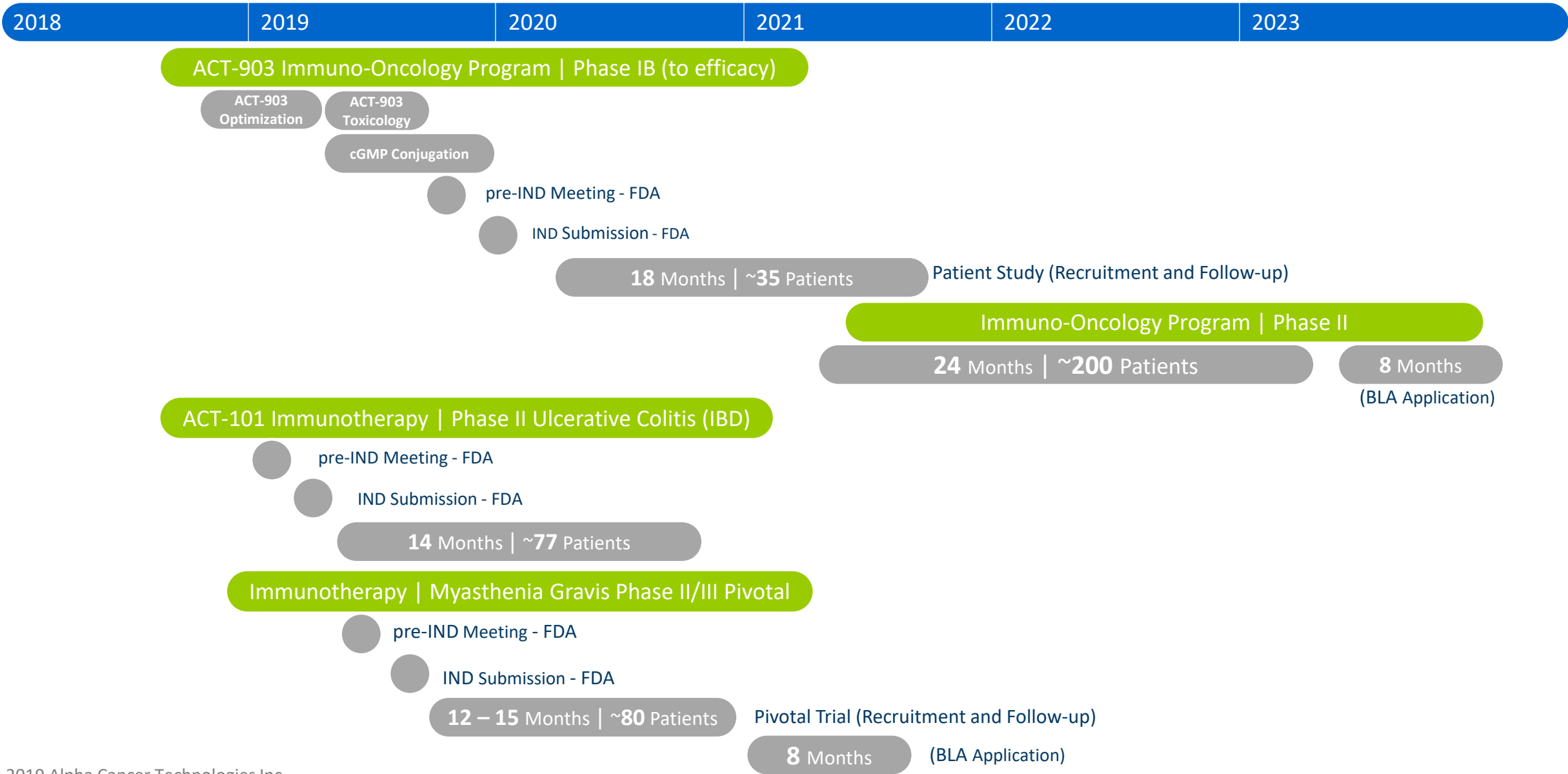
- ✓ Validated targeted chemotherapy platform, delivering chemotherapy drugs to cancer cells

MANUFACTURING

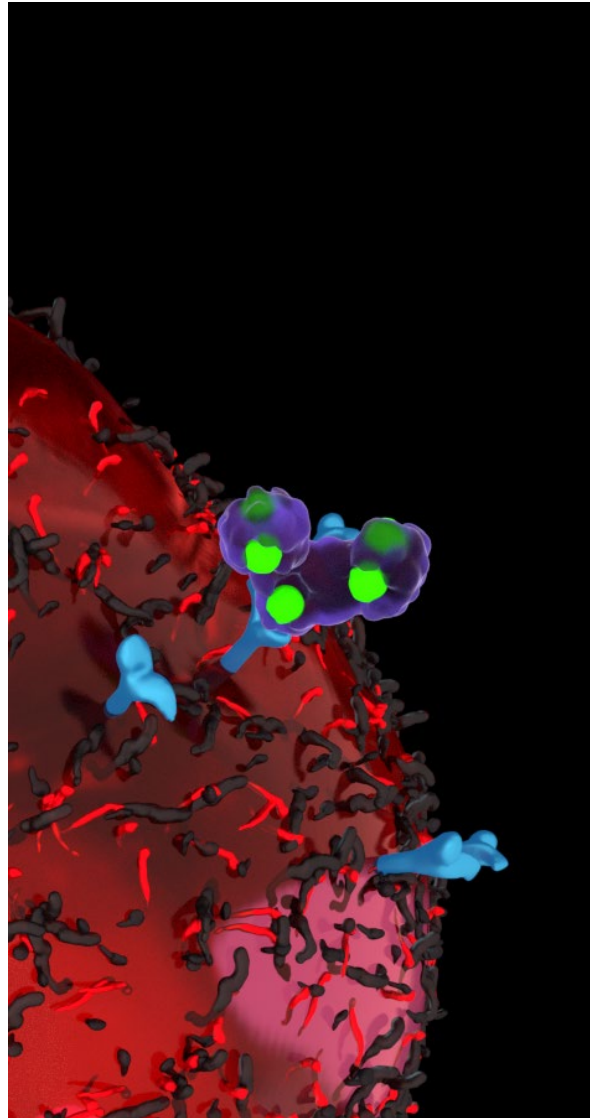
- ✓ FDA-approved manufacturing processes for both ACT-101 and chemotherapy
- ✓ Outsourced commercial manufacturing to combine ACT-101 and chemotherapy

PRICING & REIMBURSEMENT

- ✓ Will substitute for chemotherapy's established use delivering a targeted and less toxic therapy
- ✓ Competitive with other targeted therapy options
- ✓ High probability of reimbursement in target markets



BUSINESS DEVELOPMENT SUMMARY



IMMUNO-ONCOLOGY PLATFORM

- ✓ Novel targeted chemotherapy delivery platform - AFP
- ✓ Targeting protein fully characterized
- ✓ Mechanism of action well understood
- ✓ Massive safety data in over 300 humans – non-immunogenic
- ✓ International patents in place to 2025+ and new filings and additional 7/12 years market and data exclusivity as a biologic
- ✓ Receptors found on >80% of all cancers (solid and liquid)
- ✓ Receptors found on MDSC cells
- ✓ Evidence of efficacy even in drug resistant tumors
- ✓ Demonstrated superior activity at low chemotherapy dose
- ✓ FDA approved manufacturing process in place
- ✓ Significant risk mitigation and short path to value inflection- – Phase IA/B (treatment to efficacy)
- ✓ Lead assets ACT-901, ACT-902, ACT-903 - clear paths to success

CAPITAL RAISE AND USE OF FUNDS

- Currently raising up to \$15M
 - \$5M already closed; included strategic participation
 - All common shares
- Funds to be used for:
 - Phase II Myasthenia Gravis (MG) study
 - Pre-IND for IBD with ACT-101
 - Phase IB (to efficacy) ACT-903 oncology study
 - Preclinical research in multiple sclerosis
 - Manufacturing clinical supplies
 - Simultaneously working to risk/share, partner or monetize ACT-101



RISK FACTORS

Investing in our securities is speculative and involves a high degree of risk. You should consider carefully the following risk factors.

Risks Related to Our Business and the Development and Commercialization of Our Product Candidates

We have a limited number of product candidates, all which are still in preclinical or early clinical development. If we do not obtain regulatory approval of one or more of our product candidates, or experience significant delays in doing so, our business will be materially adversely affected.

We do not currently have any products approved for sale or marketing. As a result, we are not currently permitted to market any of our product candidates in the United States or in any other country until we obtain such regulatory approvals. Our product candidates are in early stages of development and we have not submitted an application, or received marketing approval, for any of our product candidates. Obtaining regulatory approval of our product candidates will depend on many factors, including the following:

- successfully completing formulation and process development activities;
- completing clinical trials that demonstrate the efficacy and safety of our product candidates;
- receiving marketing approval from applicable regulatory authorities;
- establishing commercial manufacturing capabilities; and
- launching commercial sales, marketing and distribution operations.

Many of these factors are wholly or partially beyond our control, including clinical advancement, the regulatory submission process and changes in the competitive landscape. If we do not achieve one or more of these factors in a timely manner, we could experience significant delays or an inability to develop our product candidates at all.

Successful development of our current and future product candidates is uncertain and we may discontinue or reprioritize the development of any of our product candidates at any time, at our discretion.

Before obtaining regulatory approval for the commercial distribution of our product candidates, we must conduct, at our own expense, extensive preclinical tests and clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Preclinical and clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. Success in preclinical studies or early-stage clinical trials does not mean that future clinical trials will be successful because later-stage clinical trials may fail to demonstrate sufficient safety and efficacy to the satisfaction of regulatory authorities. There is a high failure rate for drugs proceeding through clinical studies. Alternatively, management may elect to discontinue development of certain product candidates to accommodate a shift in corporate strategy, despite positive clinical results.

We face significant competition and if our competitors develop and market products that are more effective, safer or less expensive than our product candidates, our commercial opportunities will be negatively impacted.

We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies, universities and other research institutions. Many of our competitors have significantly greater financial, manufacturing, marketing, drug development, technical and human resources than we do. Specifically, there are a large number of companies developing or marketing treatments for cancer and autoimmune disorders, including many major pharmaceutical and biotechnology companies.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe effects, are more convenient or are less expensive than any products that we may develop.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since inception and anticipate that we will continue to incur losses for the foreseeable future. We have no products approved for commercial sale, and to date we have not generated any revenue or profit from product sales. We may never achieve or sustain profitability.

We are a clinical-stage biopharmaceutical company. We have incurred significant losses since our inception. We expect to continue to incur losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek regulatory approvals for, our product candidates, prepare for and begin to commercialize any approved product candidates and add infrastructure and personnel to support our product development efforts and operations. The net losses and negative cash flows incurred to date, together with expected future losses, have had, and likely will continue to have, an adverse effect on our shareholders' deficit and working capital. The amount of future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. To become and remain profitable, we must succeed in developing and commercializing product candidates with significant market potential.

We will require substantial additional funding, which may not be available to us on acceptable terms, or at all, and, if not available, may require us to delay, scale back, or cease our product development programs or operations.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is expensive. We will continue to require additional funding to complete the development and commercialization of our product candidates and to continue to advance the development of our other product candidates, and such funding may not be available on acceptable terms or at all.

Until we can generate a sufficient amount of product revenue to finance our cash requirements, which we may never do, we expect to finance future cash needs primarily through a combination of equity offerings, debt financings, strategic partnerships and grant funding. If sufficient funds on acceptable terms are not available when needed, or at all, we could be forced to significantly reduce operating expenses and delay, scale back or eliminate one or more of our development programs or our business operations.

FOR FURTHER
INFORMATION
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THANK YOU



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