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# INVESTOR SUMMARY - FULL VERSION

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*All enquiries should be directed to:*

**Edward L. Jacobs (CEO)**
**Email:** Ed.Jacobs@Ascalon.com
**Phone:** + (480) 268-2045

9977 N. 90th Street, Suite 110
Scottsdale AZ 85258
www.ascalon.com

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## Highlights

- **Ascalon finalized a CRADA (Cooperative Research and Development Agreement) with NCI/CTEP for Fenretinide.**
- **Ascalon has now established contacts with virtually all of the major oncology companies, and is in close dialogue with several with respect to specific co-development and licensing projects.**
- **Ascalon received a Discovery Grant from the Federal Government in December.**
- **Ascalon attended the JP Morgan meeting in San Francisco in January.**
- **Ascalon presented in public for the first time at OneMedForum in San Francisco in January.**
- **Ascalon is presenting at the National Venture Capital and Angel Investor Forum in New York in February.**

## Summary

Ascalon International Inc (Ascalon) was founded in mid-2009 in Scottsdale (Arizona, USA) as an oncology drug development company focused on the in-licencing or co-development of high opportunity, high value programs from both small and large pharmaceutical companies, and progressing them, faster and more efficiently, to significant commercialization exit.

Supported so far by seed money from two main sources (US Oncology and TRAC (Translational Accelerator LLC)), plus the personal investment of money and time of its founder management team, and most recently a Discovery Grant, Ascalon has progressed its business model, acquiring rights to its first product, Fenretinide. Based on the strong relationships we enjoy with pharmaceutical companies, clinicians and potential investors, Ascalon is uniquely placed to develop a valuable and reduced risk portfolio of up to 10 oncology compounds. These opportunities will be unveiled for investment purposes as they progress.

**However, following this acquisition of the global rights to Fenretinide, a program which represents a strong and near term commercial opportunity, Ascalon is now seeking a capital investment of $7.5M to progress Fenretinide to an out-licencing inflection point in 2013.**

This investment will enable Ascalon to secure an FDA SPA (Special Protocol Agreement) for three high need indications for Fenretinide, PTCL (Peripheral T-cell lymphoma), CTCL (Cutaneous T-cell lymphoma) and Upper Gastric Adenocarcinoma (UGA), based on promising phase I data. It will also allow Ascalon to progress early phase II trials to provide unequivocal evidence of a strong product positioning in at least one and potentially all three lead indications by mid-2013, which will in turn drive early acquistion of product rights by a major pharmaceutical company. It is the objective of Ascalon to secure this early term deal, consistent with the 'pay as you go' development approach we have identified for Fenretinide, and reflectant of Ascalon's philosophy of selecting opportunities with attractive market postioning and commercialisation profiles allied to highly controlled costs and focused timelines. We have also identified that to take Fenretinide to full regulatory approval for all three indications during 2015 and 2016 will involve a further capital requirement of approximately $24M. This expense would probably be structured within the out-licencing deal itself for Fenretinide as Ascalon will continue to deliver the development milestones leading to full market commercialisation.

Whatever the timing of the licensing, the overall exit rewards could be considerable. The most comparable deals are those involving Spectrum's acquisition in 2010 of belinostat, a potential PTCL target for the US, India and China, for $350M, and Celegene's purchase of Gloucester for $640M in Dec 2009, comprising $340M cash and a further $300M in future milestones, which largely reflected the value of Istodax (Romidepsin) the lead product, approved for CTCL and recently filed for PTCL. The potential profile for Fenretinide in the developing markets for CTCL and PTCL looks strong based even on phase I data, plus its potential in UGA outweighs the commercial potential in both T-cell lymphoma indications.

**Even assuming that we achieve an early deal for Fenretinide, including upfront payments and milestones, totalling only perhaps $250M, even for all three indications, and discounting its further clinical potential in other indications, this would still drive an NPV of well in excess of $60M and offer an attractive return by 2015 also well in excess of 4x any investment made.**

## Ascalon Business Model and Market Opportunity

Ascalon was founded to capitalize on the availability of promising oncology compounds to in-license, as well as the growing trend in the pharmaceutical industry for strategic outsourcing of development programs to share innovative thought, risk and reward. As there are estimated to be approximately 800 product candidates in oncology clinical development, key to Ascalon's success is its ability to access and choose the very best and most promising compounds, with minimized attrition risk, but also with the largest potential to achieve excellent return on investment.

Pivotal to this has been the ability to build a strong management team, highly experienced and capable of making product selection decisions and spearheading optimal product development or co-development through established alliances with our development partners, namely TGen/TD2, US Oncology, Parexel International and most recently the National Cancer Institute (NCI).

Also key to the model is a diligent business development and market analysis approach. Several hundred drug opportunities have already been assessed and discarded according to very deliberately and firmly applied criteria. It is also worth noting that in two instances to date Ascalon has ultimately declined involvement in major projects due to the prospective opportunities not meeting our benchmarks of attrition risk and return on investment.

However, further project options have been identified and there are on-going discussions with several major pharmaceutical companies exploring the possibilities of managing the development of select investigational drugs in their pipelines. In addition, at least five other major companies have indicated a desire to initiate dialogue. Of note, we are in advanced business discussions with one 'top five' oncology company whereby Ascalon would manage the Phase III development of a highly promising and key compound in their pipeline and where the company specifically believes Ascalon can play a key role in the strategic and operational completion of the program. Ascalon expects to financially participate in the development and to earn a return payable in milestone payments and royalties commensurate with its investment and value creation. This program, and others, will be more formally detailed for further investment purposes once potential operational plans have been progressed.

## Ascalon Progress to date

**Management Team assembled:** Ascalon has assembled a balanced and high quality senior management team (see Appendix) led by 3 executives, Dr. Dan Von Hoff, Ed Jacobs and Dick Love, each with strong pharmaceutical industry and investor credibility, backed by executives with considerable experience in the commercial, development, regulatory and international theatres. Each member has well over 20 years of extensive experience in the international biopharmaceutical and oncology industry and the team has collectively been involved with a distinguished list of blockbuster oncology products, especially our CSO, Dr. Dan Von Hoff, current holder of the ASCO Karnofsky prize. The team is working closely to identify and screen product development candidates using the strong industry and clinical relationships it collectively enjoys, as well as its close understanding of key groups like NCI, FDA and EMEA. Most recently, Dr Bill McCulloch, former CMO of Gloucester Pharmaceuticals has joined Ascalon, so enabling us to utilize his invaluable recent experience in gaining approval for Istodax in CTCL.

The operational team to drive project Fenretinide is already largely in place and can be expanded at modest cost to enable development of further products as they materialise.

**Operational Partnership achieved and communicated:** Collectively TGen/TD2, US Oncology and Parexel are a seamless and highly valued extension to the core Ascalon offering. This 'ready to go' collaboration, enabling Ascalon to oversee rapid and innovative development of promising strategic products in an attractive financial package, has already been accepted as operationally valid by major pharmaceutical groups.

**Fenretinide (N-(4-hydroxyphenyl) retinamide, 4-HPR) - project 1:** Fenretinide offers exactly the exciting opportunity and commercial potential for which Ascalon is geared for and Ascalon has secured an exclusive global license option from the innovators, CerRx. Although Fenretinide is well documented in the scientific literature it is in fact a hitherto largely unexplored drug, for various historical reasons, but one that offers a unique and highly selective mode of action towards tumor cells. (It increases intracellular ceramide levels and generates reactive oxygen species which selectively promotes apoptosis in tumor cells). Now harnessed in a proprietary nanoparticle I.V. formulation the product is now able to deliver the optimized plasma levels to achieve clinical effect, at low toxicity, and we have IP protection to at least 2021. In phase I studies Fenretinide has not only demonstrated the early clinical evidence to achieve strong adoption in rapidly developing commercial markets like CTCL and PTCL but, by its unique mode of action and low toxicity, promises both single agent and combination potential in further indications including those where it has already displayed robust early clinical activity, such as UGA, neuroblastoma, ovarian carcinoma, colorectal adenocarcinoma and B-cell lymphoma (mantle cell).

Of particular commercial note is the fact that Fenretinide has already demonstrated very significant and consistent phase I activity at intended dose levels in T-cell lymphoma patients including some refractory to Istodax. Both CTCL and PTCL are commercially emergent areas offering orphan drug protection, and where currently approved drugs show very modest activity and where there is a clear need for better therapies. Also of note is the fact that Fenretinide has shown strong activity in UGA, an increasingly prevalent tumor type in the US with high unmet need, and again offering orphan drug eligibility.

Significantly, the NCI/CTEP has expressed strong interest in Fenretinide and is collaborating with Ascalon. A draft CRADA (Cooperative Research and Development Agreement) has been approved which will enable the NCI to conduct a number of phase II clinical trials for CTCL, with additional formulation, biomarker and combination studies as needed. In addition, several cooperative study groups are expanding their studies independently and at minimal or no cost to Ascalon. The Children's Oncology Group is commencing a phase III study in neuroblastoma, and the California Cancer Consortium in association with Texas Tech is expanding their current phase I study to add additional UGA patients to validate the strong signal seen to date. Collectively, the value of the NCI and cooperative group studies are considerable in the support this provides to the core investment to Ascalon. Fenretinide already shows the potential to be a drug with sales approaching $1B, based even on the indications which we feel we can endorse through the immediate near term clinical development activity. Several pharmaceutical groups have already indicated early interest in acquiring commercialization rights to the product once it has reached a slightly more advanced status, and based on recent similar oncology transactions the return on investment made by Ascalon could be extremely attractive.

**Low Funding utilized so far:** So far Ascalon has progressed by way of maintenance funding from US Oncology and TRAC, a low burn rate, and the Therapeutic Discovery Grant. In addition we have identified other means of supporting Fenretinide through various grant sources, including the Texas Emerging Technology Fund (ETF), and the Cancer Prevention Research Institute of Texas (CPRIT) fund. ETF has awarded a $2.5 M grant to CerRx and CPRIT monies are being explored in concert with CerRx and our partner, US Oncology. However the overall conclusion should be that Ascalon has maintained an undiluted financial position which should be attractive to new investors, whether they are attracted to Fenretinide or to other yet emergent opportunities.

**Further deals potential – large and small pharma**
Ascalon is progressing further high value deals. These deals will be subject to further project specific capital raising activities, and will assume minimal G&A increase, and specific extra project management cost other than that identified in the Fenretinide development plan. Several opportunities have already presented themselves with the likely NPV and risk profiles appropriate to Ascalon.

## Project Fenretinide

Ascalon is ready to move ahead with Fenretinide according to the core assumptions defined below. A detailed commercial model, which can be shared on request, has been developed to validate these assumptions and their variables.

*Assumed Commercial Indications*

- PTCL
- CTCL
- Upper Gastric Adenocarcinoma (UGA)
- ('Other' indications (e.g. ovarian carcinoma) are not included here but to be decided based on current phase I programs )

*Timings*

- PTCL - NDA 2014, approval 2015 (also EMEA six months to one year later)
- CTCL - NDA 2014, approval 2015 (also EMEA as above)
- UGA - NDA 2015, approval 2016 (also EMEA as above)

These timings are realistic based on our assessment of the scale and design of the studies involved and the liklihood of receiving a relatively accelerated approval based on a market leading target of 50% ORR (CR + RR) which we believe could actually be a modest target based on the impressive phase I data to date.

*Development design and implementation expenses*

Detailed phase II/III trial designs are still in discussion with NCI and FDA, but for PTCL and UGA will involve direct management by Ascalon while CTCL will be managed in conjunction with NCI/CTEP. Detailed preparation and development expenses are contained in the full Fenretinide development plan but are envisaged as follows:

- Fenretinide product manufacture costs $400K
- CTCL phase II development (with NCI) $500K
- PTCL phase II development (stage IIa) $1.1M to signal validation leading to stage IIb/III expansion (further costs approximately $9M)
- UGA phase II development (stage IIa, following phase I validation) $1.5M leading to stage IIb/III expansion (further costs approximately $11M)

*Exit timing / rationale*

It is currently assumed that based on strong phase IIa data in any of PTCL, UGA or CTCL, versus incumbent therapies, that an exit agreement could be made with a major pharmaceutical company during mid-late 2013, inducing a cascade of further payments based on completion of clinical and regulatory milestones. This agreement may enable phase II/III expenditure currently budgeted within the Fenretinide development plan to be transferred onto a new partner. However, we have curently assumed this expenditure within our development plan, to be met by agreement proceeds and that Ascalon will continue with development responsibility.
Based on recent industry benchmark transactions, a relatively conservative cascade of up-front payments and milestones has been assumed at this stage within the model, comprising $75M by 2013 and another $175M in milestone payments by 2016, with royalties following that to be negotiated based on market sales. Licensor rewards and milestone payments are as contractually agreed including 10% of any exit payments and milestones.

*Product positioning, price, share and revenue assumptions- a conservative view*

Ascalon has also based its valuation model on modest assumptions with respect to market sizes, available patients and market shares achieved in the US and ex-US in each of the three indications. We have only included US and EU for consideration at this stage and have discounted, purely for simplicity, upsides in other territories and indications. In each indication a conservative second or third line position is currently assumed versus Istodax (CTCL), various minimally active treatments (UGA) and both Folotyn and Istodax in PTCL, even though phase I data alone suggests we could be more aggressive. We are also balanced on our views with respect to the relative lateness of our launch versus recently approved products such as Istodax and Folotyn. Those products will be established by the time of the Fenretinide launch but we will also benefit from their market development activities. We see parallels with other recently 'untreated' conditions like MDS (Myelodysplastic Syndrome). With MDS new products have helped develop attention and 'tendency to treat' enabling market size to expand quickly. For all three of the initial indications for Fenretinide we feel this is also likely to be true including UGA because of the greatly increasing incidence of that cancer in the US. But for the sake of revenue assumptions here we have held our market sizes at very conservative levels with low growth rates within the base model. Treatable US and non-US market sizes are so far assumed as broadly equal with respect to each of the three indications as is the evolution in patient treatment access and prevalent patient pool size over time. Fenretinide price is also assumed at a very competitive $14k per course and we are assuming an average of five courses per treatment except with UGA (where there is assumed to be four). This pricing is at least 20% lower than that for both Folotyn and Istodax to support rapid formulary and managed markets adoption.

According to the valuation differentiators listed above there are many scenarios that can be envisaged for Fenretinide revenue. However our favoured 'realistic' scenario with the following base, key assumptions provides the revenues and valuation in table 1 below:

- **CTCL** – specific treatable patient population 4000 pts (2010, 50% US), treatment cost $70k, market share maximum (after 7 years) 30%.

- **PTCL** – specific treatable patient market 14000pts (2010, 50% US), treatment cost $70k, market share maximum (after 7 years) 30%.

- **UGA** – specific treatable patient population 12600 pts (2010, 50% US) (this is perhaps especially conservative ex-US), treatment cost $56k, market share maximum (after 6 years) 30%

Potential downsides to product revenue flow exist such as delay in approval, worse than expected product positioning providing lower share, lower price and slower than expected market evolution. However we believe upsides such as better pricing, and much higher market treatable market sizes and shares due to strong positioning versus incumbent products are far more likely and will be recognised by a licencing partner.

*Corporate investment assumed*

A base cost assumption has been made for corporate structure at this stage, considered base and sufficient to enable project support focused on Fenretinide plus further basic business development and project diligence capability.

*Success probabilities and other variables*

At Ascalon not only do we believe that we can improve upon currently accepted product attrition levels within the oncology drug development industry by better program design, but that we will be able to streamline project development costs and timelines. It is possible to insert 'probability success' levels into the current Fenretinide model to envisage secondary project valuations. However based on where we are, with extremely promising phase I data, already showing potentially good positioning versus competitors in CTCL and PTCL, we feel that other factors which could also be classified as upsides such as higher than expected milestones, royalties, product price, market size, market share, development of other indications and the non-emergence of generic pressure from 2022 due to IP extension can also be equally considered. All these factors and variables can be demonstrated on request to benchmark against valuations developed by potential investors.

*Project revenues and investment returns*

**As previously stated the potential revenues shown below above are driven by conservative assumptions as is historically appropriate for drug development in the cancer sector. However for the purpose of this document we are still able to suggest that revenue projections could reach in excess of $800M by the end of the 2015-2022 time period. Therefore, a conservative deal worth $250M in upfront payments and milestones staggered between 2013 and 2016, would drive an NPV of well in excess of $60M and a return on investment of well in excess of 4x capital.**

| $M | 2011-2012 | 2013-2014 | 2015 | 2016 | 2017 | 2018 | 2019 | 2020 | 2021 | 2022 |
|---|---|---|---|---|---|---|---|---|---|---|
| **Sales CTCL** | 0 | 0 | 17.9 | 46.9 | 65.7 | 78.8 | 88.2 | 97.6 | 101.3 | 105.1 |
| **Sales PTCL** | 0 | 0 | 24.0 | 77.0 | 160.1 | 222.1 | 281.2 | 313.8 | 343.0 | 361.0 |
| **Sales UGA** | 0 | 0 | 0 | 37.9 | 104.6 | 183.0 | 240.6 | 298.0 | 345.2 | 376.5 |
| **Total Sales** | 0 | 0 | 41.9 | 161.8 | 330.4 | 483.9 | 610.0 | 709.4 | 789.5 | 842.6 |

Table 1 Projected Fenretinide revenues based on current model assumptions

## Appendix

Ascalon Management Team

Founded by prolific leaders in oncology drug development, Ascalon has assembled an experienced management team, each with over 20 years of experience in the biopharmaceutical industry. Ascalon's founders and management team have collectively been involved in a long and distinguished list of blockbuster oncology drugs.

| Name | Title | Experience |
|---|---|---|
| Richard L. Love | Chairman | TRAC, ILEX, Parexel Board of Directors |
| Daniel Von Hoff, MD | Chief Scientific Officer, Director | TRAC, TGen/TD2, US Oncology, ILEX |
| Edward L. Jacobs | President, CEO & Director | SuperGen, Johnson & Johnson, Adria Labs |
| William McCulloch, M.B., Ch.B. | Regulatory Consultant | Gloucester Pharmaceuticals, A.M. Pappas, Sparta Pharmaceuticals, US Bioscience |
| Mark Lewis | Senior Vice President, International Operations | SuperGen, Chiron, Quintiles, Servier, Farmitalia, Amersham |
| William J. Simpson | Vice President, Business Development | AstraZeneca, Adria Labs, Wyeth |