Aptorum Group Limited (NASDAQ:APM) Investor Update Call Transcript
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Corporate Participants

Mr. Ian Huen
Founder, Chief Executive Officer and Executive Director

Mr. Darren Lui
President and Executive Director

Dr. Clark Cheng
Chief Medical Officer and Executive Director, Aptorum Group Limited
Executive Director, Aptorum Medical Limited

Darren Lui

Ladies and gentlemen, good day, and welcome to the Aptorum Group Limited Quarter 1 2020 Investor Update Conference Call. My name is Darren Lui and I am the President and Executive Director of Aptorum Group.

As a reminder all participant lines will be on listen-only mode. Please note that this conference is being recorded and the replay will be available on our website afterwards. The call transcript will also be put up on our website shortly.

Before we begin, I would like to remind you the various remarks that we make on this call may contain forward-looking statements, subject to risks, uncertainties and other factors that may cause actual results to differ materially from those expressed or implied. All forward-looking statements made on this call are made based on the beliefs of Aptorum Group Limited as of this date only. Future events or simply the passage of time may cause these beliefs to change. For a more detailed description of the risks that impact these forward-looking statements, please refer to our annual reports on Form 20-F. Please be aware that you should not place undue reliance on the forward-looking statements made today.

Our update today will be split into 4 main topics:

1. Our latest therapeutic initiative on COVID-19;

2. Latest update on existing pipeline on SACT-1 (targeting Neuroblastoma) and ALS-4 (targeting Staphylococcus Aureus);

3. Our current progress on commercialization of our NLS-2 women's health supplement; and

4. Finally, we will address some of the recent key questions received from our investors.

I will now hand over to our Chief Executive Officer and Executive Director, Mr. Ian Huen. Thank you and over to you, Ian.

Ian Huen

Welcome to this call for providing an update to all our stakeholders on certain recent development of Aptorum Group.

First of all, regarding our latest strategic initiative on the Covid-19 disease:

1) COVID-19 disease is highly contagious and confirmed cases globally have now exceeded 800,000[1] and the World Health Organization has declared COVID-19 as a global pandemic. There are a number of pharmaceutical companies developing vaccine based therapies to protect people from catching the virus. However, we think the therapeutic standpoint for treating sick people is equally important, if not more, and we must deliver a fast response to COVID-19.

   In order to accelerate development, as announced recently, we have initiated an additional research and development project targeting the coronavirus group and have completed initial screening under the Smart-ACT™ platform, which is our drug repurposing discovery platform. Out of a library of more than 2,600 approved small drug molecules, we have identified at least 3 potential candidates (collectively "SACT-COV19") for further preclinical investigation against the new coronavirus, COVID-19. The benefit of our strategy is that the identified repurposed drug candidates have already undergone human trials that established the drugs safety, toxicity and pharmacokinetic clinical profiles, thus potentially significantly shortening the development costs and timeline to reach human clinical trials for the new indication. To immediately commence this work, as announced, we have also entered into collaboration with The University of Hong Kong's Microbiology Department to

[1] https://www.arcgis.com/apps/opsdashboard/index.html#/bda7594740fd40299423467b48e9ecf6

conduct further preclinical testing of the selected candidates before submitting for regulatory clearance for clinical trials. The University of Hong Kong is extremely well known for the discovery and vaccine work of the SARS virus in 2003 and currently the University is also playing a major part of the CEPI initiative to develop a vaccine and support the government's initiatives in monitoring the COVID-19 virus.

Secondly, regarding our latest update on our existing therapeutic pipeline:

1) for our ALS-4 project, a small drug molecule candidate indicated for the treatment of infections caused by Staphylococcus aureus (or "S. aureus"), including methicillin-resistant Staphylococcus aureus (commonly known that MRSA), is based on a novel anti-virulence non-bactericidal approach. Through inhibiting the production of staphyloxanthin, ALS-4 renders Staph aureus highly susceptible to the host's immune defense. This novel mechanism is significantly different from the bactericidal approach found in currently marketed and developing antibiotics used to treat Staph. aureus, which are experiencing increasing drug resistance issues. Specifically, MRSA infections in humans typically exhibit high rates of morbidity and mortality and can cause complications such as infective endocarditis, pneumonia or sepsis, with relapse, and as a result hospital readmission. Staph aureus bacteremia infection is very common and treatment can be extremely costly. Therefore, a number of these indications are currently targeted in our clinical trials plan.

   In February, we released further positive data from our current investigational new drug (IND)-enabling studies. As elucidated in our previous press release dated September 9, 2019, ALS-4 did not show any mutagenicity in the in vitro Ames tests. Our currently generated in vitro micronucleus test results also showed that ALS-4 is not genotoxic, indicating the non-mutagenic nature of the drug. Furthermore, the results of the in vitro hERG assay study predicts a low risk of ALS-4 causing cardiac QT prolongation. We have also demonstrated the efficacy of ALS-4 in animal models upon oral administration. Compared with the current mainstay of treatment for MRSA infections such as vancomycin or daptomycin which is typically administered in an IV injectable form (with the exception of an oral form vancomycin specifically for treatment of Clostridium difficile diarrhea and staphylococcal enterocolitis only), an oral active agent enables wider market penetration targeting both outpatient and potential prophylactic markets. Subject to completion of the current studies, Aptorum Group targets to submit IND for ALS-4 in the second half of 2020 to commence phase 1 trials in North America.

2) Another update is for our SACT-1 program. SACT-1 is the first repurposed drug candidate to be developed under the Smart-ACT™ drug discovery platform, which employs a systematic approach to screen a library of over 2,600 market approved small molecule drugs in order to identify, patent and repurpose against a currently identified universe of 7000+ (and increasing) orphan diseases, over which over 90% is still untreated or does not have effective treatment. Through the Smart-ACT™ platform, Aptorum Group has successfully identified a patentable candidate: SACT-1, which shows preclinical efficacy for the treatment of neuroblastoma, being an entirely new therapeutic area from its currently approved indication. Recently, we announced significant progress about the positive data and development in relation to the repurposed SACT-1. Neuroblastoma is a rare type of childhood cancer that develops in infants and young children. Our current pre-IND preparation is going well and we are on course to apply for orphan disease designation and file an IND under the 505(b)(2) pathway in the second half of 2020.

Thirdly, regarding current progress on commercialization of our NLS-2 women's health supplement:

1) Our dietary supplement, NLS-2, is targeted for the relief of women undergoing menopause or post-menopausal cycles and experiencing related symptoms such as flushes and even osteoporosis. The supplement is made with Chinese yam (dioscorea opposite) powder containing an active ingredient called "DOI", which is Aptorum Group's non-hormonal approach intended to meet certain growing $17bn[2] per year global women's health consumer nutritional market. We have entered into a regional distribution agreement with Multipak Limited, a local company holding the famous tea brand "Luk Yu" and other health products. The supplement will initially be sold in Hong Kong and we are seeking regulatory clearance to market the product in other major jurisdictions. At this stage, the production of Aptorum Group's NLS-2 DOI based bioactive nutraceutical tablets has commenced production in Canada and will be marketed under the brand name NativusWell™. The product is expected to be revenue generating in 2020.

I will now hand it over to Mr. Darren Lui.

[2] Isoflavones market size to reach USD50.06bn by 2025.
https://www.grandviewresesarch.com/press-release/global-isoflavones-market.

Darren Lui

Thank you very much, Ian. Well, we have received and consolidated a number of questions from our investor community over the last few days and our Chief Medical Officer and Executive Director, Dr. Clark Cheng, and myself will now address these questions.

Q1) For what shall the proceeds from the recent capital raise of USD10m in February 2020 be used?

We attracted a strong round of interest from US institutional funds and existing shareholder interests in our company through the February capital placement. The proceeds will be utilized for a number of areas, including the following:

a) Firstly, the expansion of our drug development pipeline, including the continuing development of our Smart-Act platform of SACT-2, SACT-3 and the recently announced SACT-COV19 projects;

b) Secondly, the continued development of our existing leading therapeutic candidates of SACT-1 and ALS-4 to their respective clinical phases targeted in second half of 2020;

c) Thirdly, the continued commercialization of our NLS-2 women's health supplement; and

d) Finally, potential acquisitions of additional technologies that will enhance our stakeholder value, however, we currently do not have specific commitments or agreements for any such acquisitions.

Q2) What are the key catalysts in Q2 and second half of 2020?

The key catalysts for the remainder of the 2020 year will include the following:

a) our pending progress in developing SACT-1 and ALS-4 into the respective clinical stages in North America; specifically, it is targeted that SACT-1, as a repurposed drug with an established safety, toxicity and PK record in the past, will pursue the 505b(2) regulatory pathway and subject to FDA's clearance to commence neuroblastoma Phase 2 related trials in 2020; ALS-4 also subject to IND application, will be targeted to commence safety related phase 1 trials in North America in second half of 2020;

b) towards quarter 2 2020, we hope to report back our progress of our preclinical studies for our SACT-COV19 therapeutic candidates and any further collaboration parties for this project; subject to the further readout of our preclinical studies, we will update the investor community on further timelines to proceed towards clinical trials and will explore with the relevant regulatory agencies for any fast track review processes if available;

c) Thirdly, towards the remainder of the year, we also expect our NLS-2 supplement for women's health is on track and is expected to commence generation of revenue for the Company through our proprietary distribution and third-party distribution channels; and,

d) Finally, we are constantly reviewing opportunities for strategic investments and acquisition opportunities, there are however no existing commitments or agreements in place; we will report back to our investors in quarter 2 2020 and make the appropriate disclosures to market if this becomes relevant.

Q3) What is the clinical development plan for SACT-1 and ALS-4?

I will now hand over to our Chief Medical Officer and Executive Director Dr Clark Cheng to answer this and the next few questions.

[Clark Cheng]

Thank you Darren. For ALS-4, we seek to add our drug to the standard of care, which is IV vancomycin or daptomycin for the treatment of MRSA infections. Indeed, the mortality of MRSA infections is still around 30-40% even though the standard of care is given, indicating the current treatment cannot adequately address the unmet needs in this disease area. We see to use ALS-4, which is not an antibiotic, to overcome the challenges experienced by the existing antibiotics, namely, high mortality, long duration of infections, high relapse and recurrence rates and these will also be our clinical end-points in our clinical trials. Our first indication will be targeting a small patient population, for example, a subgroup of MRSA bacteremia, to enable us to file NDA with FDA via a LPAD pathway which stands for Limited Population Pathway for Antibacterial and Antifungal Drugs. In parallel, we will also run clinical trials on other general indications such as pneumonia, skin and soft tissue infections, urinary tract infections, etc. The clinical assessment of ALS-4 in prophylaxis of MRSA infections will also be conducted.

For SACT-1, we will be targeting the high-risk patient group, especially those who fail to respond to any available treatment. We are planning our clinical trials for SACT-1 both as a single drug and as a combination therapy to the standard of care, subject to the trial results, and we will assess for overall and progression-free survival as the primary end-points, tumor response rate as our clinical end-points in our trials.

Q4) Where are our key research development facilities and sites?

Our key research development facilities are located primarily in two sites: the first one is in Hong Kong Science Park where we have access to over 2,700 sq ft of laboratory space primarily focused on preclinical work including formulation, CMC and non-GLP related preclinical preparation;

Our second site is in Toronto where in partnership with Covar Pharmaceutical, we carry out GMP manufacturing through over 4,000 sq ft of laboratory space; our Toronto site also helps to coordinate our planned GLP and clinical trials for our SACT-1 and ALS-4 in North America.

Q5) What is the market size of those indications (e.g. Neuroblastoma, Staph. Aureus) and why are these indications considered unmet?

The global market size of Neuroblastoma in 2017 was already over USD 2.6 billion and the expected global market size by 2023 will be around USD 3.23 billion. The disease Neuroblastoma is considered unmet because children in the high-risk group have a 5-year survival rate of around only 40% to 50%[3] even though standard of care is given, which is usually chemotherapy plus radiation and other drugs such as isotretinoin, etc. In our preclinical study, SACT-1 shows synergistic efficacy with chemotherapy and our repurposed drug has a well-established safety profile in humans, up at 150mg/day, the death rate was 0% in prior clinical studies with no dosage related adverse events[4]. We strongly believe our SACT-1 add-on to the standard of care would be beneficial to neuroblastoma patients, especially in the high-risk group.

The global market size of Staphylococcus aureus infections including MRSA in 2016 was USD

[3] https://www.cancer.net/cancer-types/neuroblastoma-childhood/statistics

[4] Page 6 / 7 of http://ir.aptorumgroup.com/static-files/66346f79-7a03-474a-89be-0eaafaa00d9d

2.97 billion and the expected global market size by 2025 will be USD 3.91 billion[5]. MRSA is considered unmet because the current treatment cannot adequately address the medical need. The mainstay of treatment for MRSA infections is usually IV vancomycin or daptomycin. However, the mortality is still as high as 40% (in Pneumonia cases) and 60% (in Bacteremia cases)[6] even when the drug is given and development of complications such as endocarditis, pneumonia and relapse and recurrence is still common. With our impressive preclinical data, we are attempting to add ALS-4 to the standard of care to overcome the challenges that the existing antibiotics are experiencing. As shared early on, we plan to use ALS-4 to address 3 clinical endpoints including (i) reduction in mortality rate, (ii) reduction of the duration of infections and (iii) reduction of relapse and recurrence. In addition, as mentioned earlier, contrary to the injectable forms of the existing antibiotics such as vancomycin or daptomycin, ALS-4 is the orally active and bioavailable form of ALS-4 and in the outpatient setting for prophylaxis market should not be overlooked.

Q6) What are our thoughts on Covid-19?

Covid-19 is now a global pandemic and have already exceeded 800,000 confirmed cases globally. We are concerned that given the potential mutation of the SARS-COV-2 virus strain and the infection behavior, this disease may well continue to last for the medium term and also provide additional challenges of the effectiveness of upcoming vaccines being developed. Although the current SARS-COV-2 virus (current mortality rate 4.1%)[7] has a lower mortality rate than that of the 2003 SARS-COV-1 virus strain (mortality rate 9.6%)[8] or the 2012 MERS-COV virus strain (mortality rate 37%)[9], the current SARS-COV-2 virus has a more resilient survival period and higher infection rate. So it is now our strategy to tackle this disease from a therapeutic standpoint and we will continue to investigate and develop our SACT-COV19 drug candidates under our Smart-ACT platform for the treatment of COVID-19, and hope to work with our collaborator to speedily deliver a workable therapeutic solution to the world. We will continue to provide our stakeholders update of this progress in the next few months.

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[5] MRSA Drugs Market – Global Industry analysis, Size, Share, Growth, Trends and Forecast, 2017-2025 (2018)

[6] https://www.ncbi.nlm.nih.gov/books/NBK482221/

[7] www.worldometers.info. Archived from the original on 31 January 2020 and retrieved 2 February 2020.

[8] Smith, Richard D. (2006). "Responding to global infectious disease outbreaks: Lessons from SARS on the role of risk perception, communication and management". Social Science & Medicine. 63 (12): 3113–3123.

[9] https://www.mdpi.com/2076-0817/9/3/231/htm

I will now hand back over to our President and Executive director, Mr Darren Lui for the remaining questions.

Darren Lui

Thank you Dr Clark Cheng. I will continue to answer the remaining questions.

Q7) What were the factors causing recent volatility in the stock price?

In our view, the recent downturn generally in U.S. markets is mainly attributed to the global economic downturn caused by the spread of the Covid-19 disease, which has become a global pandemic as declared by the World Health Organization. We would like to inform our stakeholders that despite the virus situation, our development progress and recent expansion of pipeline continue to operate on a business-as-usual basis and have not been affected by the recent COVID-19 pandemic. With the recent capital raise completed and supported by our US institutional fund shareholders, our fundamentals remain strong and we will continue to target to deliver those key catalysts and drive shareholder value as we have planned and communicated to our investors so far.

Q8) What is the current cash position and how long will this sustain the development?

After our February capital raise, as of mid-March 2020, our current cash position, including our undrawn credit line is over USD20m. This will continue to fund our operations and any strategic initiatives well over the next 12 months and will continue to help drive our lead candidates into the various clinical trial stages.

Q9) When is the next major update?

We expect to provide ongoing major updates via our press release updates such as business wire and our virtual investor update arranged by Solebury Trout as our IR agent. The next virtual update will be held on April 2nd, 2020 and details are available on our investor relations website. If you would like to be added to our newsletter distribution list, please feel free to email your contacts to investor.relations@aptorumgroup.com. Our second quarter 2020 investor telephone conference update will be targeted for June 2020 and further information will be announced closer to date.

I shall now hand over to our Chief Executive Officer and Executive director, Mr. Ian Huen, for

closing remarks.

Ian Huen - Closing

On this note I would like to conclude by saying that year 2020 is a key pivotal year for Aptorum Group as a number of our programs progress towards clinical stages especially in the second half 2020 and if all goes as planned, the above explained new strategic projects are expected to add significant value to the Company.

On behalf of the Company's executive management, we thank everyone once again for joining the call. And if you have any further questions, we would be happy to respond to you and please send your questions to investor.relations@aptorumgroup.com. We look forward to communicating with all of you again next quarter.

On behalf of Aptorum Group Limited, that concludes this conference. Thank you for joining us and you may now disconnect your lines. Thank you.