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# Interim Phase III Results for PI-88

**Brisbane, Australia, 28 July 2014.** Progen Pharmaceuticals Ltd ("Progen" or "the Company") (ASX: PGL, OTC: PGLA) today announces the results of the interim analysis for the Phase III PATRON clinical trial for PI-88.

The Company's PI-88 licensee, Medigen Biotechnology Corp. ("Medigen") (Taipei, Taiwan), has today disclosed the results of the interim analysis of PI-88's Phase III PATRON clinical trial. The PATRON trial is a fully recruited randomised, placebo-controlled Phase III trial being conducted in Taiwan, South Korea, China and Hong Kong to confirm the safety and efficacy of PI-88 in the adjuvant treatment of hepatocellular carcinoma after surgical resection.

The interim analysis conducted by an independent committee of medical and statistical experts is based on the results from the first 131 recurrent patients (representing 60% of the total targeted recurrent 218 patients). The results have indicated that PI-88 has a good safety profile, and that based on recurrent data from each individual clinical trial centre, PI-88 did not meet the primary endpoint of Disease Free Survival. Further data analysis by the independent committee will be performed when data from BioClinica, an independent medical imaging company in the US, is available.

In 2010, Progen licenced the worldwide oncology rights of PI-88 to Medigen to complete product development and commercialisation of PI-88.

Further information on the clinical trial protocol and the statistical analysis used for the interim analysis is available in the attached appendix.

**ENDS**

**About Progen Pharmaceuticals Ltd**
Progen Pharmaceuticals Limited is a biotechnology company committed to the discovery, development and commercialization of small molecule pharmaceuticals primarily for the treatment of cancer. Progen has built a focus and strength in anti-cancer drug discovery and development. www.progen-pharma.com

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This release contains forward-looking statements that are based on current management expectations. These statements may differ materially from actual future events or results due to certain risks and uncertainties, including without limitation, risks associated with drug development and manufacture, risks inherent in the extensive regulatory approval process mandated by, amongst others, the United States Food and Drug Administration and the Australian Therapeutic Goods Administration, delays in obtaining the necessary approvals for clinical testing, patient recruitment, delays in the conduct of clinical trials, market acceptance of PI-88, PG545, and other drugs, future capital needs, general economic conditions, and other risks and uncertainties detailed from time to time in the Company's filings with the Australian Securities Exchange and the United States Securities and Exchange Commission. Moreover, there can be no assurance that others will not independently develop similar products or processes or design around patents owned or licensed by the Company, or that patents owned or licensed by the Company will provide meaningful protection or competitive advantages.

## APPENDIX

Additional information of the clinical trial protocol and the statistical analysis used for the interim analysis is provided below.

### Study Title

A prospective, randomized, double-blind, placebo controlled, parallel-group, international multicenter phase III trial of PI-88 in the adjuvant treatment of subjects with hepatitis virus related hepatocellular carcinoma after surgical resection.

### Clinicaltrials.gov Identifier

NCT01402908.

### Study Phase

Pivotal Phase III Trial.

### Study Objectives

Primary Objectives: To evaluate the efficacy of daily administration of PI-88 versus placebo for the adjuvant treatment of study subjects as measured by disease free survival (DFS) during the study period. The primary endpoint of this study is the DFS during the study period. The DFS is defined as time, in weeks, from randomization to tumor recurrence or death due to any cause during the study period.

Secondary Objective: The secondary objectives are to evaluate (a) safety and (b) efficacy, as measured by time to recurrence (TTR), overall survival (OS), tumor recurrence (TR) rate.

*Note: the secondary objectives were not included in the interim analysis by the Sponsor.*

### Methodology

Number of Subjects: The study will aim to recruit 500 subjects who were randomized to two treatment groups in a (treatment vs. placebo) 1:1 ratio. The treatment group received 160 mg PI-88 via subcutaneous injection for four consecutive days per week, for 3 weeks out of every 4 weeks. The control group also received placebo via subcutaneous injection for four consecutive days per week, for 3 weeks out of every 4 weeks.

Study Population: Adult male and female subjects with hepatitis B or C viral infection and histologically-proven hepatocellular carcinoma, having had curative resection within 4-6 weeks prior to randomization. The curative nature of the resection is to be confirmed by follow-up chest and abdominal CT scans and abdominal MRI scan within 4 weeks prior to randomization.

Duration of Treatment: The maximum time on study drug for each subject is 52 weeks. Subjects will continue to be followed up for another 96 weeks.

### Statistical Considerations

An approximate total of 500 subjects, PI-88: placebo = 250:250, were enrolled to observe 218 events of tumor recurrence or death from any cause among the subjects. The interim analysis now reported herein is based on 131 events (tumor recurrence or death from any cause) in the intent-to-treat (ITT) population which includes all subjects who were randomized onto the study. Based on the previous phase II clinical study result (Liu *et al*, 2009), this sample size will be sufficient to reject the null hypothesis that the PI-88 and placebo survival curves are equal with 85% power and type I error at two-sided 0.05.The underlying assumptions are median DFS time on the placebo is 12 months and PI-88 treatment shall increase median DFS on PI-88 to 18 months.

## Reference

Liu CJ, Lee PH, Lin DY, Wu CC, Jeng LB, Lin PW, Mok KT, Lee WC, Yeh HZ, Ho MC, Yang SS, Lee CC, Yu MC, Hu RH, Peng CY, Lai KL, Chang SS, Chen PJ (2009) Heparanase inhibitor PI-88 as adjuvant therapy for hepatocellular carcinoma after curative resection: a randomized phase II trial for safety and optimal dosage. *J Hepatol*. 50(5):958-68. doi: 10.1016/j.jhep.2008.12.023.