



First patients dosed in Phase 2a proof-of-concept study with rectal formulation of TopiVert's TOP1288 in moderate to severe ulcerative colitis

London, UK, 6 October 2016: TopiVert Pharma Ltd ("TopiVert" or the "Company"), a clinical-stage biotechnology company developing Narrow Spectrum Kinase Inhibitors (NSKIs) as novel, locally-acting medicines for the treatment of chronic inflammatory diseases of the gastrointestinal (GI) tract and eye, announced today that the first patients have been dosed in its Phase 2a proof-of-concept study to evaluate the safety, tolerability and efficacy of a rectal formulation of TOP1288 in symptomatic ulcerative colitis (UC) patients with moderate to severe disease activity.

TOP1288, the Company's lead NSKI programme, is a potent inhibitor of key kinases involved in inflammation and it is being developed by TopiVert as a topical therapy for UC, a form of inflammatory bowel disease (IBD) which affects the colon. As many as 40% of patients fail to respond to current drug therapy, which is often poorly tolerated, and some patients require surgical removal of the colon in order to manage the disease.

TopiVert reported encouraging results from a Phase 1 study of a rectal formulation of TOP1288 in healthy volunteers earlier this year¹, and this has subsequently been supported by data from an additional patient cohort. These clinical data, which include encouraging biomarker responses and negligible systemic absorption, support the concept that TOP1288 has the potential to produce sustained effects in mucosal tissues after local administration, but without the undesirable side effects often seen in UC patients treated with systemically available therapies.

The ongoing Phase 2a study with TOP1288 is a randomised, double-blind, placebo-controlled study to evaluate the efficacy, safety and tolerability of TOP1288 administered once daily for four weeks in symptomatic UC patients with moderate to severe disease activity. The study, which aims to recruit up to 60 patients at sites across eight European countries, is expected to report in the second half of 2017. The current study utilises a rectal formulation of TOP1288, to help demonstrate POC, and a Phase 1 study with an oral formulation of TOP1288, as the intended commercial presentation, is on track to commence in early 2017, with results also anticipated in the second half of 2017.

Professor Simon Travis, Professor of Clinical Gastroenterology at the University of Oxford and Principal Investigator, commented: *"Ulcerative colitis affects the lives of over 2 million people in Europe and the USA, at a cost of over \$4 billion a year, and we are seeing a rapid rise in cases in the Gulf, Indian subcontinent and East Asia that mirrors what happened in the Western world fifty years ago. Current therapies do not work or are poorly tolerated by many patients. TOP1288 has shown very promising data and I am excited by its potential to improve the treatment for patients with ulcerative colitis."*

Ajay Duggal, CMO of TopiVert, commented: *"After such positive data in Phase 1 with TOP1288 earlier this year, we look forward to seeing whether TOP1288's potent anti-inflammatory effects translate, in*

the current proof of concept study, into clinical benefits for patients with active ulcerative colitis. With both a Phase 1 study for an oral formulation of TOP1288 in ulcerative colitis and a first-in-human proof-of-concept study for TOP1630 in dry eye disease planned for early 2017, TopiVert is set to deliver important clinical data from several key studies in the second half of 2017.”

¹Rowley, A; Duggal, A; Taylor, M; Foster, M; Solanke, Y; Sirohi, S; Walshe, C.; Webber, S; Irving, P.; Travis, S. (2016). A First-in-Human Randomized Double-Blind Placebo-Controlled Clinical Trial of a Novel Narrow Spectrum Kinase Inhibitor. *Gastroenterology*. Volume 150, Issue 4, Supplement 1. Pages S776-S776.

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About TopiVert

TopiVert is a clinical-stage biotechnology company developing narrow spectrum kinase inhibitors as novel, locally-acting medicines for the treatment of chronic inflammatory diseases of the gastrointestinal tract and eye. The Company's most advanced drug candidate, TOP1288 for the treatment of ulcerative colitis, is currently in Phase 2 development, with a Phase 2a proof of concept study due to report in the second half of 2017. TopiVert also expects to start the clinical development of TOP1630, its candidate for dry eye disease (DED), in early 2017. Current therapies for these debilitating diseases provide inadequate long-term control in a high proportion of patients and considerable unmet medical need remains. The Company commenced operations in early 2012 and its investors include Imperial Innovations, SV Life Sciences, NeoMed and Johnson & Johnson Innovation-JJDC, Inc.

About Narrow Spectrum Kinase Inhibitors (NSKIs)

NSKIs are novel small molecules characterised by relatively broad, potent anti-inflammatory activity and only minimal systemic exposure. Specifically, NSKIs are potent inhibitors of a select range of pivotal kinases involved in inflammatory cascades of both innate and adaptive immunity. Simultaneous targeting of multiple inflammatory components leads to a synergistic activity profile with broad anti-inflammatory effects. The NSKIs are designed to have low bioavailability to reduce their exposure to many of the body's healthy tissues, thereby enhancing their safety and tolerability profiles. Together, these attributes make NSKIs ideal treatment candidates for chronic inflammatory diseases where long term therapy demands a sustained effect accompanied by excellent safety and tolerability.

About IBD and ulcerative colitis

Inflammatory Bowel Disease (IBD) is a term used to describe several diseases that involve inflammation of the gastrointestinal tract. The two most common forms of IBD, Crohn's disease and

ulcerative colitis, together affect over 4 million people worldwide. They are both chronic relapsing conditions that cause bloody diarrhoea, abdominal pain and significant reductions in a patient's quality of life. While their causes are not fully understood, these diseases are characterised by an abnormal inflammatory reaction that leads to damage of the intestinal wall.

Current treatments for ulcerative colitis involve administration of oral, rectal or intravenous/subcutaneous anti-inflammatory and immunomodulatory therapies, including biologics. Despite these products being effective in treating active disease in some patients, their long term use is often hampered by safety and tolerability issues. Furthermore, at least 40% of patients have poorly controlled disease and around 20% require surgery to manage the disease.