TopiVert reports successful Phase I study with TOP1288 for the treatment of ulcerative colitis

- *Excellent safety and tolerability profile with minimal systemic uptake supporting NSKI product concept*
- *TopiVert to present the data at the 11th Congress of ECCO in Amsterdam, 18 March 2016*
- *Phase II proof of concept study to start mid-2016*

London, UK, 17 March 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 tract and eye, today announced positive Phase I study results for TOP1288, its lead compound for the treatment of ulcerative colitis (UC). TopiVert will present the Phase I study data at the 11th Congress of ECCO in Amsterdam on Friday 18 March 2016.

The Phase I study results demonstrate that a rectal formulation of TOP1288, a potent inhibitor of key kinases involved in inflammation, has an excellent safety and tolerability profile in healthy volunteers when administered once or twice daily for five days at a dose of up to 200mg. Furthermore, TOP1288 was detected in tissue biopsies taken from the colon 24 hours after dosing, positive signals of target engagement and biomarker response were observed and only minimal drug was found in the systemic circulation. These results support the concept that NSKIs have 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 current systemically available therapies.

UC is a relatively common inflammatory disease affecting the colon and approximately 40% of patients fail to respond to current drug therapy, which is often poorly tolerated. The disease can be serious enough to require surgery to remove the colon in some patients.

TopiVert plans to start a Phase II proof of concept study with a rectal formulation of TOP1288 in UC patients in mid-2016, with results anticipated by mid-2017. This study is designed to demonstrate whether TOP1288 provides a clinical benefit in UC patients with active disease while avoiding significant exposure to healthy tissues outside the gastrointestinal tract.

TopiVert is also planning to start a Phase I study of an oral formulation of TOP1288 in the second half of this year which will similarly report in mid-2017. An oral product offers greater patient acceptability and convenience over a rectal formulation.

Dr Steve Webber, Chief Scientific Officer of TopiVert, said: *"We are delighted that our first clinical study with an NSKI product demonstrated minimal systemic exposure when delivered locally, resulting in an excellent safety and tolerability profile. We are now eager to see whether the potent anti-inflammatory effects observed with TOP1288 in preclinical testing translate into clinical benefits for patients with ulcerative colitis in a proof of concept study."*

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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, has successfully completed Phase I development and a Phase IIa proof of concept study is planned to commence in the middle of this year. 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.

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, as many as 40% of patients remain uncontrolled and around 20% require surgery to manage the disease.