Information note on the use of a new agent, bedaquiline, for the treatment of multidrug-resistant tuberculosis

On December 28, 2012, the U.S. Food and Drug Administration approved bedaquiline, a new therapeutic agent, as part of combination therapy to treat adults with multi-drug resistant tuberculosis when other alternatives are not available. Other national regulatory authorities in the world are currently in the process of evaluating this compound as well.

Tuberculosis (TB), one of the world’s deadliest diseases, is caused by Mycobacterium tuberculosis. It is mostly spread from person to person through the air and usually affects the lungs, but it can also affect any other organ of the body. According to WHO, 8.7 million people around the world became sick with TB in 2011. Multi-drug resistant TB (MDR-TB) occurs when the mycobacterium becomes resistant to isonazid and rifampin, the two most effective anti-TB drugs commonly used to treat TB. MDR-TB results from either infection with organisms which are already drug-resistant or may develop in the course of a patient’s treatment. According to WHO, there were an estimated 310 000 MDR-TB cases among notified pulmonary TB patients in the world in 2011.

It is acknowledged that currently available data on the safety and efficacy of bedaquiline arise from Phase IIb trials, and that further efficacy and safety data will be needed from rigorously conducted Phase III trial(s). Based on current, publicly-available data on the efficacy and safety of this compound, as well as on supplementary analyses being requested from independent experts, WHO is initiating a review process aimed at developing rapid interim guidance on the potential use of bedaquiline for the treatment of MDR-TB. An expert review meeting will be convened for this purpose at the end of January 2013.

The latest WHO guidance on MDR-TB treatment was issued in 2011 and can be found at: http://www.who.int/tb/challenges/mdr/programmatic_guidelines_for_mdrtb/en/index.html. These guidelines present the WHO recommendation for optimal treatment of MDR-TB using combinations of standard anti-TB drugs. If a treatment regimen is failing, the recommended practice is to add multiple drugs informed by Drug Sensitivity Testing or by data from setting-specific Drug Resistance Survey(s). In an effort to maximise the likelihood of successful treatment outcome and prevent acquisition of additional resistance, thus preserving the use of the drugs for future generations of TB sufferers, WHO advises that a single drug deemed to be effective should never be added alone to a regimen to which a patient is not responding to. This is particularly relevant now and in the coming months as new anti-TB drugs like bedaquiline will be released on the market.