

Preface

Example of Recent Success in Drug Treatment (1)

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Just 10 years ago, Chronic Myeloid Leukemia (CML) was a universal killer. All forms of treatments made little effect on the cause of disease. They only controlled the white cell count, platelet count and the size of the spleen, but had little effect on the natural evolution or the course of the disease. Most patients survived few years and almost none was alive at 9 years. The only known "curative" treatment at the time was allogeneic Bone Marrow Transplantation (BMT). Even in the best centers, the long term survival results were in the range of 50-60%. Given the limitation in the availability of donors and the age of patients, only 20% of patients could be offered this form of therapy.

When imatinib (glivec) was discovered to inhibit the tyrosine kinase domain of the BCR-ABL (the abnormal fusion gene which causes the disease), clinical trials followed (such as the IRIS trial), comparing the results of imatinib with those of the best treatment available at the time which was alfa interferon with cytosine arabinoside.

It was soon found that major improvements were documented in all aspects of the disease, especially the effect on the abnormal Philadelphia chromosome and the gene causing the disease. For the first time, a new treatment made major impact on the BCR-ABL gene and caused the disappearance of the fusion gene in the great majority of patients. As a result, patients with CML can now live normal life for many years without evidence of disease.

With the publication of long-term results of the IRIS trial, the complete molecular and cytogenetic effect has been maintained in more than 85% of patients after six years. It is projected that patients will probably survive 20 years or more disease-free. This represents a "functional" cure.

The drug proved to be safe and is given orally. However, the yearly cost of this treatment is quite

high and may be beyond the budget of many of the developing countries. Some patients may be intolerant to the drug and some others may develop resistance through single base mutations or through other means. Despite of all this, it is unethical today not to give this drug to a newly diagnosed patient with CML.

Before imatinib, CML was a major indication for BMT in selected patients. Its role is now limited to patients who acquire specific types of mutations and have a matching donor and are in the right age group.

The success of this drug has created a plethora of research of new "targeted" drugs to treat all forms of cancers. I expect that within the coming 5 to 10 years we will see many new medications which will achieve some sort of significant success against certain types of cancers. This will change the way we practice this treatment and the way we treat our patients. With this in mind, we have to be ready to spend substantially more on these drugs. We need, as a developing country relying heavily on health tourism, to negotiate with major pharmaceutical companies to get some financial support for our patients to receive these new medicines. One way of doing that is to expand on research with participation in large trials and exploring new ways to generate loco-regional trials and investigations.

The scene is changing rapidly and we have to adapt to it rather than set and complain about how "greedy" these companies are. When a major break through in therapy is available we, as medical doctors, cannot but use it for the best of the interest of our patients.

After all, we are ethically and medico-legally obliged to advice our patients on the best treatment available.

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