## **Platinum Priority**

Reply from Authors re: Camillo Porta. How to Identify Active Novel Agents in Rare Cancers and then Make Them Available: A Need for a Paradigm Shift. Eur Urol 2012:62:1020–1

Research in Rare Tumors: A Need for a Paradigm Shift

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We thank Professor Porta for his thoughtful comments [1] about the implicit issues raised by our report [2] of sunitinib therapy for a heterogeneous mixture of rare subtypes of kidney cancer. We could not agree more that the academic oncology community must find a more efficient paradigm for the treatment of rare cancers. Moreover, we believe that the most productive path toward this goal is to embrace Bayesian notions in the context of adaptive trials that incorporate candidate biomarkers. We have previously championed these ideas in the context of the phase 2 selection problem [3–5] and see ample justification for extension to rare cancers, as described by Porta [1].

One interesting extension of these ideas would be to establish registries of rare cancers. Even though this "uncontrolled" experience has well-known pitfalls, it is probably our best hope for obtaining "prior distributions" to support the design of targeted trials. We need a way to pool experience and "mine" that experience for hypotheses that can be investigated more formally.

By whatever specific mechanisms, there is a moral obligation to learn something from every patient we treat, particularly regarding rare cancers, for which there will probably never be data from large phase 3 trials. The comments of Porta [1] are extremely important and most welcome.

Conflicts of interest: The authors have nothing to disclose.

## References

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http://dx.doi.org/10.1016/j.eururo.2012.08.016

DOIs of original articles: http://dx.doi.org/10.1016/j.eururo.2012.06.043, http://dx.doi.org/10.1016/j.eururo.2012.07.035

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