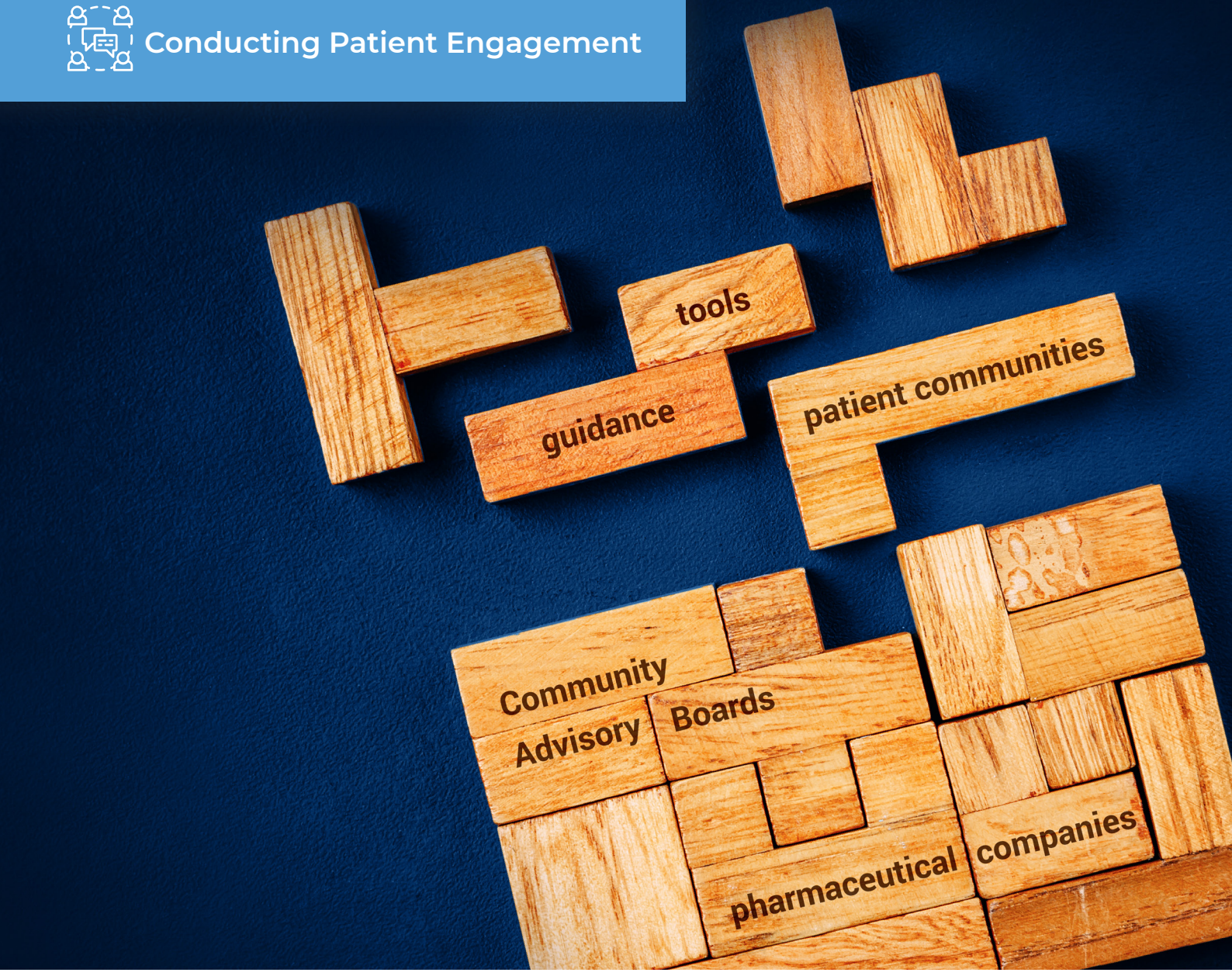
PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

innovative medicines initiative

Conducting Patient Engagement



Community Advisory Boards

Tool 8: Examples of successful outcomes of CABs and industry interactions

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Examples of successful outcomes of CABs and industry interactions

Example from the ECAB (EATG):

The pharmaceutical developer Tibotec (now Janssen Therapeutics) designed two studies in 2005. The DUET phase III trials involved the concurrent use of two compounds in a HIV-treatment experienced population.

The unique feature of the trial was that both compounds used, had not been licensed at the time of use (2006). This was the first occasion that two unlicensed compounds were used in a trial in a treatment experienced setting, albeit only in one arm, while the other arm of the trial remained placebo controlled. HIV infection is an incurable but manageable disease that requires a relatively rigorous regime of antiretroviral medication (ART) for patients in order to avoid resistance. Resistance to certain drugs or classes of drugs is more common with treatment experienced patients who therefore need novel or more complex regimens to control virus reproduction in the body.

The ECAB played a key role in achieving the result that a trial –for the first time– involved the concurrent use of two unregistered compounds. The standard procedure is to use a single new compound in a trial. The objective of this intervention of the patient community was to make sure that a potent novel combination of ART is available as salvage therapy for heavily treatment-experienced patients. Compassionate use of the novel treatment regime through the trial was advocated for.

Consultation between ECAB, other patient communities and the pharmaceutical developer matured and evolved during this process significantly. The patient organisations involved could successfully demonstrate to the industry and the regulators that the knowledge and experience of the patient community can yield substantial input into the development process.

The innovative approach of the community infused the development process with a certain degree of “courage” to apply unconventional strategies when preliminary results from previous trials are convincing enough (both new compounds were already known to be safe and well tolerable at the time).

This new approach led to lasting results and trust between the stakeholders involved. Collaboration between European and US CABs entered a new, more intensive phase, thus

allowing exchange of experience across the communities of people living with HIV. The DUET study resulted in overcoming accumulated multi-drug resistance for many heavily treatment-experienced patients.

(for further information about this work, please visit: EUPATI case report)

Examples from EuroCAB (programme led by EURORDIS):

The **Cystinosis CAB** discussed with a gene therapy company the urgent necessity for their children not to stop their current successful therapy two months before administration of the gene therapy. After some reflection, the company sent the CAB a revised protocol where the previous therapy did not need to be stopped before day 0 of the new trial.

The **Duchenne CAB** has a list of concerns that they discuss with the majority of companies around clinical study protocols and making these both more efficient and more patient- and family- friendly. Some of these were:

- the use of outcome measures that are relevant to patients and caregivers and impact quality of life;
- reducing the burden of clinical studies by concentrating on what is important instead of what might be “nice to know”;
- stressing the importance of including non- ambulant patients in clinical studies or creating an extra arm for this population;
- the overuse of biopsies as well as the burden involved;
- the necessity for extensive use of a placebo (longer than 24 weeks) rather than using natural history data as a comparator.

These discussions proved fruitful and have led to some successes over the past two years: reduced number of biopsies in three separate cases; reduced the number of tests and questionnaires in several studies; reduced the length of placebo to 1 year in one study, 6 months in another and improved the randomisation ratio to placebo from 1:1 to 2:1, and in one case 3:1.

The Duchenne CAB has convinced the majority of companies they work with to share placebo data after the study, and induced them to consider making individual patient data available to families after the trial ends.