International INitiative For Outcomes for vitiligo (INFO for vitiligo)

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Background

- Vitiligo: the most common depigmentation disorder
- Over 50 years of research and 100 interventions tested
- Lack of consensus as to the outcome measures
- No cure and no firm clinical recommendations

Why outcomes are important?

- 85% of research money is wasted:
  - Important outcomes not measured
  - 50% of planned outcomes not reported
  - Published research fails to set the study in the context with all previous similar research

Systematic reviews on outcomes measures in vitiligo trials

- 25 different outcomes reported in 54 trials
- Repigmentation assessed by patients: only 4% of trials
- 17% of trials asked for patient opinion on treatment efficacy
- 96% reported repigmentation BUT 48 different scales were used and none was validated

¥ ELEFTHERIADOU et al. Which outcomes should we measure in vitiligo? Results of a systematic review and a survey amongst patients and clinicians on outcomes in vitiligo *BJD* 2012; 164 (4): 804-814

Survey amongst clinicians & patients (UK)

- Cosmetically acceptable repigmentation rather than %
- Quality of life
- Maintenance of gained repigmentation
International Initiative for Outcomes for vitiligo (Miami 2013)
Establishing International consensus on outcomes for vitiligo (target lesions)

- International consensus on core outcomes measures for target lesions:
  - Step 1 COMPLETED!: define the domains-core outcomes set
  - Step 2: define scales to measure the domains (outcomes)
  - Step 3: implementation in all future trials for vitiligo
Step 1: defining core outcomes set

- Centre of Evidence Based Dermatology
- Steering group: Dr V. Eleftheriadou, Profs. K. Ezzedine, M. Picardo, K. Thomas and A. Taieb
- 101 participants:
  - International Federation of Pigment Cell Societies (IFPCS) (European, American, Asian, and Japanese)
- 24 countries:
  - Algeria, Australia, Austria, Bahrain, Belgium, Brazil, Canada, Colombia, Egypt, France, Germany, Greece, India, Italy, Mexico, Morocco, Netherlands, Saudi Arabia, Spain, Taiwan, Tunis, United Arab Emirates, United Kingdom, and United States of America.
Step 1: core outcomes set for future vitiligo trials

- **Essential outcomes** (relevant to all interventions for vitiligo and to be reported in all clinical trials):
  - repigmentation
  - side effects
  - maintenance of gained repigmentation

- **Recommended** (to be reported if relevant to the intervention being tested and trial design used)
  - cosmetic acceptability of the results
  - quality of life
  - cessation of spreading of vitiligo
  - tolerability/burden of treatment
Step 2: scale for repigmentation

- Aim: unified scale to measure repigmentation and its characteristics
- Objective vs. subjective
- For TARGET LESIONS
International e-Delphi consensus on repigmentation measurement

- e-Delphi and face to face meeting at IPCC, Singapore (2014/2015)
- 94 participants: 61 dermatologists, 21 patients/carers, 5 journal editors, 7 researchers (response rate of 94%, from 25 countries).
- No agreement on the best outcome measure for repigmentation for target lesions. WHY?
Challenges of measuring repigmentation

- Repigmentation: %, what is considered success, target lesion vs overall (RCT vs clinical practice)
- Difficult to measure (anatomical sites, size, shape, patchy, assess to software, tools, devices)
- Pattern of repigmentation
- Skin tone
- Visible sites vs. non visible (visible sites are associated with higher psychological morbidity leading to reduction in acceptability of results)

Pre-conference workshop in Rome (Vitiligo International Symposium)

- International experts in research and clinical practice
- Face to face meeting (31 clinicians from 5 different continents)

Suggestions:
- Accurate % to measure repigmentation rather than quartiles
- Colour match ? not to be assessed
- End point to measure “successful” repigmentation
Repigmentation in vitiligo

Start of Treatment  <20% repigmentation  25% repigmentation  40% repigmentation  50% repigmentation

65-70% repigmentation  75% repigmentation  80% repigmentation  90% repigmentation  100% repigmentation
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- Sound methodology, based on experience and success of other groups#
- Patients, journals editors, representatives of regulatory authorities are actively involved
- Workshop amongst patients to be conducted in Detroit (June 2017)
- Asking the right questions
- Repeat e-Delphi (?)
