Overview of childhood visual impairment and blindness

EU Regulatory Workshop - Paediatric Investigation Plans in Ophthalmology
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Outline

Burden and causes of visual childhood impairment and blindness

Epidemiological perspective on areas of unmet needs in therapeutic trials
Burden of disease
Natural history
Outcomes
Aetiology

Epidemiology to inform paediatric ophthalmology investigation plans
Paediatric ophthalmology – potential data sources in EU

- Disorder-specific population-based prevalence or incidence studies
Paediatric ophthalmology – potential data sources in EU

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• General population-based studies (e.g., birth cohort studies)
• General population-based census or disability surveys
Paediatric ophthalmology – potential data sources in EU

- *Disorder-specific* population-based prevalence or incidence studies
- *General* population-based studies *(eg birth cohort studies)*
- General population-based census or disability surveys
- *National registers of visual impairment*
- Disability and special needs registers
Paediatric ophthalmology – potential data sources in EU

• *Disorder-specific* population-based prevalence or incidence studies
• *General* population-based studies (*eg* birth cohort studies)
• General population-based census or disability surveys
• National *registers of visual impairment*
• Disability and special needs registers
• Surveillance schemes (*eg* British Ophthalmic Surveillance Unit)
• Disease/disorder-specific registers & surveillance programmes (*eg* congenital anomaly monitoring systems, EUROCAT)
Paediatric ophthalmology – potential data sources in EU

- *Disorder-specific* population-based prevalence or incidence studies
- *General* population-based studies (*e.g.* birth cohort studies)
- General population-based census or disability surveys
- *National registers* of visual impairment
- Disability and special needs registers
- Surveillance schemes (*e.g.* British Ophthalmic Surveillance Unit)
- Disease/disorder-specific registers & surveillance programmes (*e.g.* national congenital anomaly monitoring systems)
- Multi-disciplinary visual impairment teams
- Community-based rehabilitation programmes
- Service-linked databases / registers
Incomplete picture of childhood visual impairment

• few *disease-specific* national prevalence or incidence studies

• few reliable routine national sources of incidence data eg national registers of sight impairment - *under-ascertainment and imprecision*

• limited *recent* national population-based data on causes

• difficult to assess secular trends

*non-comparability of data from different sources due to variation in taxonomies (visual impairment and aetiology)*
UNICEF  Child 0 – 15 years

WHO:
Dual anatomical and aetiological taxonomy for ‘causes’ VI / BL

Visual impairment (VI) < 6/18, LogMAR 0.5 in better eye

Severe visual impairment and blindness (SVI/BL) < 6/60, LogMAR 1.0 in better eye
Global: 1.2 million blind (BL) children
(50% in very low income countries, prevalence c. 15 / 1000)

In high income / industrialised populations:

Blindness 0.5 / 1000

Visual impairment 1.5 / 1000
British Childhood Severe Visual Impairment / Blindness Study (BCVIS)

through the
British Childhood Visual Impairment Study Group

Aim:
To determine incidence and cause(s) of severe visual impairment or blindness in children in the UK

*Lancet* 2003;362:1359-65

Supported by British Council for Prevention of Blindness; Children Nationwide Medical Research Fund; National Eye Research Centre
BCVIS – METHODS

Population-based, prospective, observational study: national active surveillance undertaken
British Ophthalmological Surveillance Unit (BOSU)
British Paediatric Surveillance Unit (BPSU)
for one year (2000)

All children <16 years in UK newly diagnosed as severely visually impaired or blind due to any disorder
BCVIS - Results

439 children newly diagnosed as SVI/BL in 2000

- 54% Male
- 72% White, 16% South Asian, 4% Black, 7% Other
- 76% additional non-ophthalmic disorder(s) or impairment(s) - ‘SVI/BL plus’
- 24% low birth weight (<2500g)
- 40% in most socio-economically deprived quintile

• 10% died within a year of diagnosis (Infant mortality in SVI/BL 20x greater than general UK population)
BCVIS: Incidence SVI/BL in UK in 2000

Annual age specific (rate)

Cumulative (lifetime risk)

per 10,000

< 1 / by 1
1 - 4 / by 5
5 - 15 / by 15

4.0
0.3
0.06
5.3
5.9
### Annual Incidence of SVI/BL

**0-15 years in UK, 2000**

<table>
<thead>
<tr>
<th>Age</th>
<th>Number of Cases</th>
<th>per 10,000 per year (95%CI)</th>
</tr>
</thead>
<tbody>
<tr>
<td>All</td>
<td>$420^1$</td>
<td>0.35 (0.31, 0.38)</td>
</tr>
<tr>
<td>SVI/BL Plus</td>
<td>328</td>
<td>0.27 (0.24, 0.30)</td>
</tr>
<tr>
<td>SVI/BL Isolated</td>
<td>90</td>
<td>0.08 (0.06, 0.09)</td>
</tr>
</tbody>
</table>
### Annual Incidence SVI/BL 0-15 years by ethnic group and sex

<table>
<thead>
<tr>
<th>Category</th>
<th>Incidence per 10,000 per year</th>
<th>Relative Rate (95% CI)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Ethnic group†</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>White</td>
<td>0.23 (0.21, 0.26)</td>
<td>Base</td>
</tr>
<tr>
<td>South Asian</td>
<td>1.2 (0.92, 1.49)</td>
<td>5.1 (3.9, 6.7)</td>
</tr>
<tr>
<td>Indian</td>
<td>0.5 (0.2, 0.8)</td>
<td>2.1 (1.2, 3.6)</td>
</tr>
<tr>
<td>Pakistani or</td>
<td>1.6 (0.1, 2.1)</td>
<td>6.7 (4.9, 9.1)</td>
</tr>
<tr>
<td>Bangladeshi</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Black</td>
<td>0.5 (0.3, 0.8)</td>
<td>2.3 (1.4, 3.7)</td>
</tr>
<tr>
<td>Other</td>
<td>0.7 (0.4, 0.9)</td>
<td>2.9 (1.9, 4.3)</td>
</tr>
<tr>
<td><strong>Sex</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Female</td>
<td>0.3 (0.28, 0.37)</td>
<td>Base</td>
</tr>
<tr>
<td>Male</td>
<td>0.4 (0.32, 0.42)</td>
<td>0.9 (0.7, 1.1)</td>
</tr>
</tbody>
</table>

† Great Britain only
BCVIS - anatomical site(s)

in 23% >1 site affected

*inc ant seg dysgenesis, multiple colobomas

** Inc albinism
BCVIS: anatomical site by other impairment/disorder

Whole globe
Glaucoma *
Cornea
Lens
Uvea
Retina***
Optic nerve **
Cerebral / v pathways
Others

* p <0.05, ** p<0.01, *** p <0.001
25%

SVI / BL
is preventable or treatable with current knowledge
BCVIS: Potentially preventable causes of SVI/BL
7.5% (33)

- Trauma: 1.0%
- Non-accidental injury: 2.0%
- Autosomal dominant disorders: 1.3%
- Rubella: 0.2%
- ROP: 3.0%
BCVIS: Potentially treatable causes of SVI/BL 17.1% (75)

- Hydrocephalus/ICP: 3.4%
- Infection: 2.5%
- Retinoblastoma: 0.5%
- Diabetes: 1.4%
- Optic neuritis: 1.4%
- Uveitis: 3.0%
- Glaucoma: 4.6%
- Cataract: 0.5%
Implications for intervention studies

- Two ‘target’ populations of SVI/BL children – ‘isolated’ or ‘plus’ non-ophthalmic impairments / disorders
- Incidence varies with age, presence of non-ophthalmic impairments, ethnicity and birthweight, socio-economic status
- CVI ‘main’ cause, but disorders are diverse, patterns (anatomical sites and aetiology) vary by presence of non-ophthalmic impairments, birthweight and ethnicity
- Scope for further primary and secondary prevention limited with current knowledge
- Require novel treatments eg for CVI
Gaps in knowledge (1)

Population of children with visual impairment (vs SVI/BL)

- Incidence unknown - likely to be at least twice that of SVI/BL
- Pattern of ‘causes’ unknown - likely to differ significantly from SVI/BL

*Implications for planning intervention studies*
Gaps in knowledge (2)

Long-term outcomes / natural history

Limited information about
- long term visual function / morbidity
- social (eg education, occupation) impact
- general and mental health
- quality of life (or other patient-reported outcomes)

of affected individuals and families

Implications for assessing impact of interventions
Gaps in knowledge (3)

Health economics of childhood visual impairment

- Limited work on ‘costs’ (financial and opportunity) and benefits of different treatment and prevention strategies

*Implications for resource allocation (research and care/services)*
Thank you!

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